



# CORPORATE PRESENTATION

4 JUNE 2019

**MEDIVIR**  
SCIENCE WORKING WONDERS

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# Medivir – Recent events

- Clinical Development focus on oncology
  - Birinapant/Keytruda® : completion phase I study – Q4 2018
  - Birinapant/Keytruda® : start of phase II study – Q4 2018
  - MIV-818: Start of phase I study – Q4 2018
  - MIV-828: CD nomination – Q4 2018
- Fixed cost base reduced with two thirds
- Strong management and cost-effective virtual organization

# 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

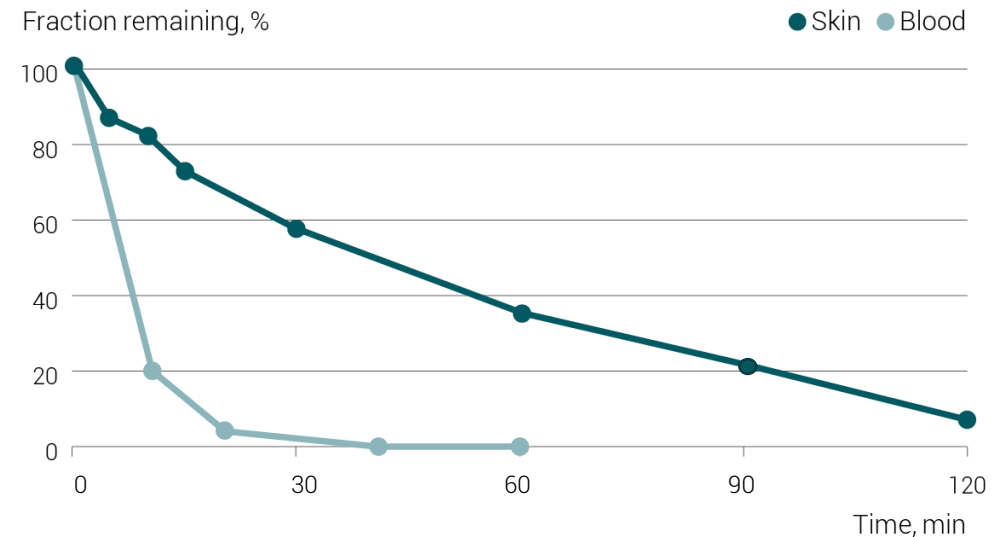
# MF-CTCL: orphan blood cancer indication

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, including systemic HDAC inhibitors, 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

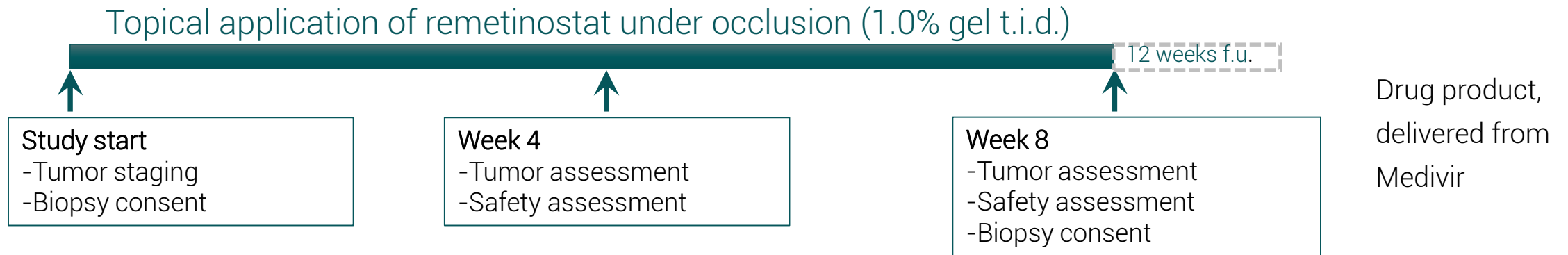
- Medivir will further define a planned phase III design based on the requirements clarified by the FDA
- One phase III study expected to be sufficient for FDA
- Phase III study will enroll treatment-experienced patients
- Medivir aims to identify a business partner for the further development of remetinostat

# Retinostat opportunity in other skin cancers

# Investigator-initiated phase II study in BCC ongoing

## 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



## Study design

- 8-week open-label phase II clinical study to assess efficacy of remetinostat in patients with BCC
- Primary objective: Determination of suppression of BCC growth & ORR (overall response rate) using RECIST v1.1 criteria

Investigator: Kavita Sarin, Stanford University School of Medicine in California, USA

# Interim results presented at SID Annual meeting May 2019

- The ORR, at least a 30% decrease in longest diameter, was 64% (9/14).
- The average decrease in tumor area is 70% (n=14), while the average decrease in longest diameter is 62% (n=14). 43% (6/14) of tumors were fully cleared.
- No systemic toxicities have been observed.
- Grade 2 eczematous local site reactions occurred in 71% (10/14) tumors treated with topical remetinostat under bandage occlusion.

More information can be found at [www.medivir.com](http://www.medivir.com)



# 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® 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

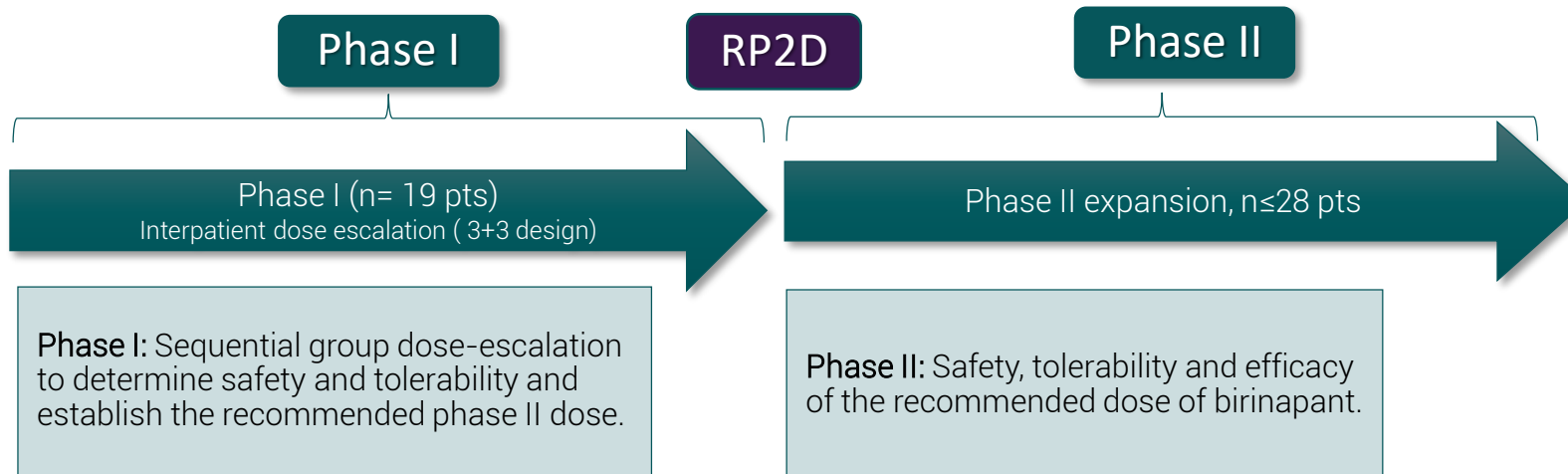
- Ovarian cancer, the leading cause of mortality due a gynecologic tumor
  - Estimated new cases 2018: US: ~ 22,000; EU: ~ 23,000 Sweden: ~ 700
  - Five-year survival: 47%
- Cervical cancer, the third most common cancer in women world-wide
  - Estimated new cases 2018: US: ~ 13,000; EU: ~ 60,000; Sweden: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

# Birinapant may benefit patients with inadequate response to immuno-oncology therapies

- Birinapant, a 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 study in solid tumors
  - Joint development committee oversees the study
  - Keytruda® provided at no cost by Merck
  - Medivir retains full global rights to birinapant and data


# Birinapant/Keytruda<sup>®</sup> combination - phase I/II study ongoing

- Dose escalation completed; December 2018: n=19
  - One CRC patient has achieved partial response, which had been maintained for over 1 year
  - Two patients had stable disease for 18 weeks
  - Safety and tolerability: No concerns
  - Phase II dose selected at 22 mg/m<sup>2</sup>



- In late December 2018 the first patient was dosed in the phase II part of the study





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

# Liver cancer focus: hepatocellular carcinoma (HCC) and intrahepatic cholangiocarcinoma

- 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

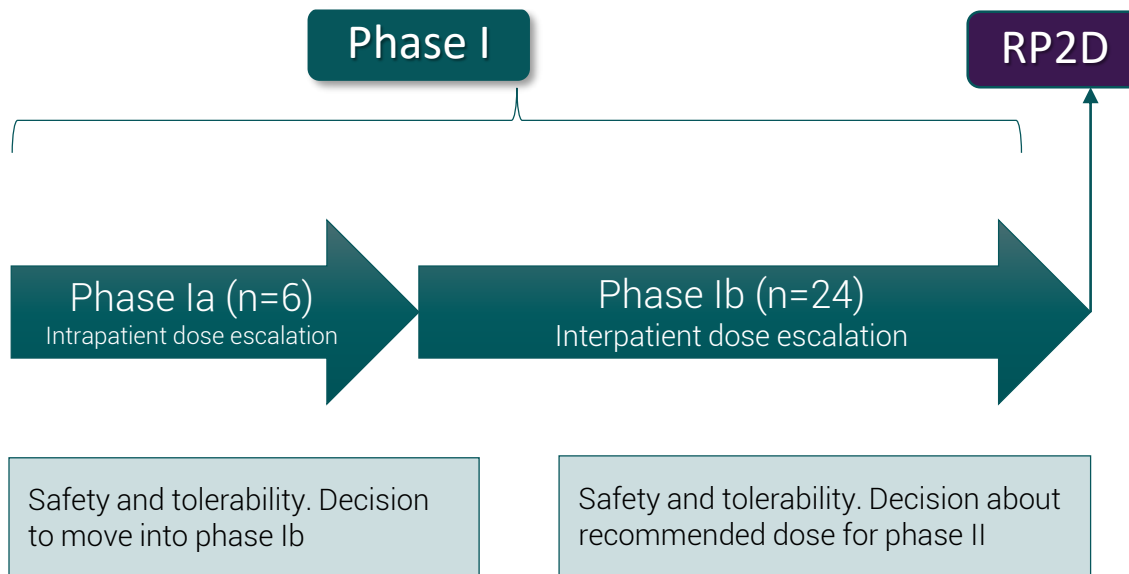
## Troxacitabine

- 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 effect



# 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

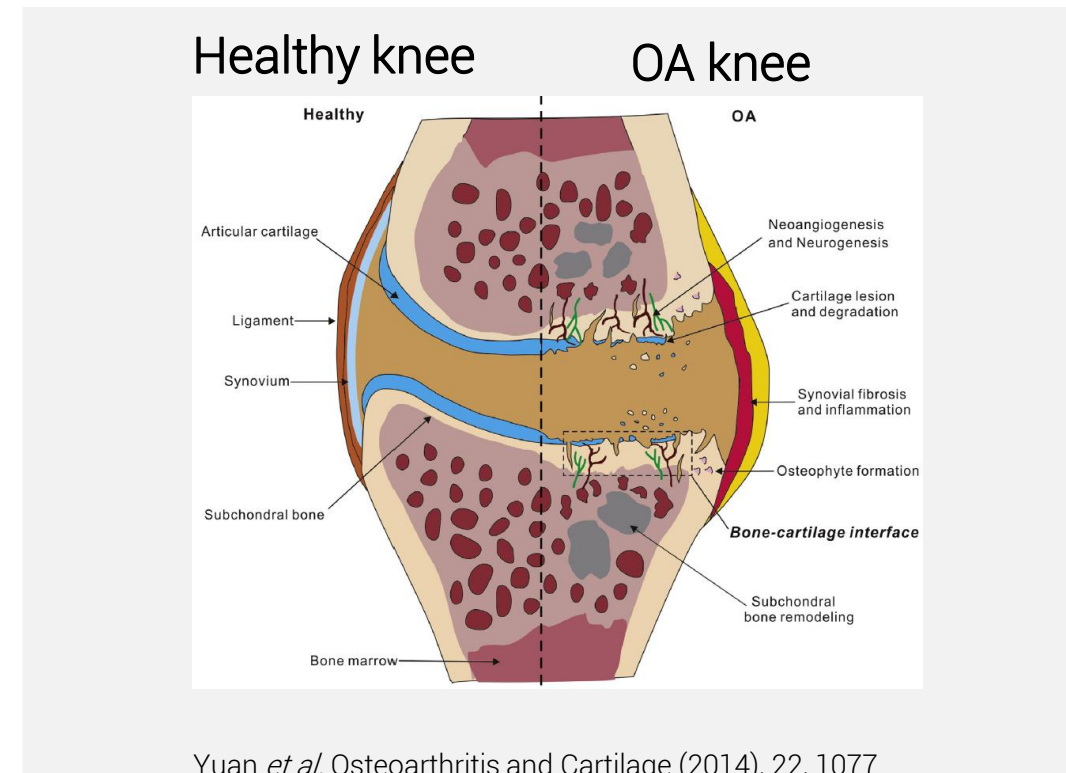


**MIV-711:  
Cathepsin K  
inhibitor with FDA  
fast track status**

# 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



# Summary

# Strategic focus on cancer indications with high unmet need

## Near term value inflection points

- MIV-818: completion phase Ia study – Q2 2019
- Birinapant/Keytruda<sup>®</sup>: fertility analysis completed – Q4 2019