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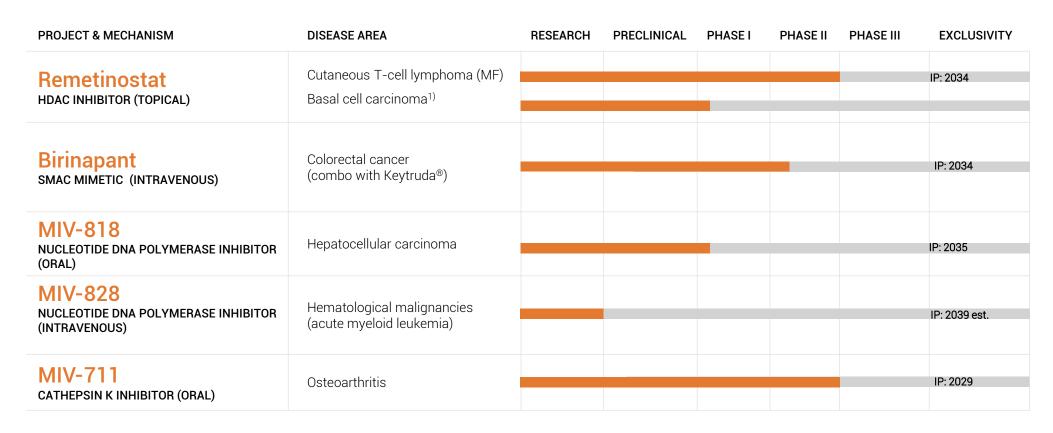


# Medivir develops transformative medicines

- Broad pipeline with four candidate drugs in clinical development
- Focus on cancer indications with a high level of unmet medical need and large commercial potential
- World-wide rights to all programs
- Near-term value inflection points
- Strong management and cost-effective virtual organization



# Broad and robust pipeline



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



# Remetinostat for early-stage cutaneous T-cell lymphoma



# MF-CTCL: orphan blood cancer indication

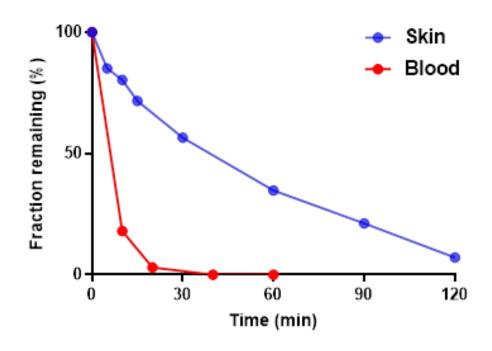
Cutaneous T-cell lymphoma (CTCL) affects lymphocytes (cells belonging to the immune defense system) located in the skin and typically has a chronic course.

- 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%	0.5%	1%
	1x/day	2x/day	2x/day
	n=20	n=20	n=20
Lesion responses <sup>1</sup>	20%	25%	40%
Patients with clinically significant pruritus	1%	0.5%	1%
	1x/day	2x/day	2x/day
	n=8/20	n=6/20	n=10/20
	(40%)	(30%)	(50%)
Pruritus responses	37.5%	50%	80%

### Well tolerated:

- No HDAC inhibitor-associated systemic adverse events
- Median time on treatment: 332 days (1% 2x/day dose)

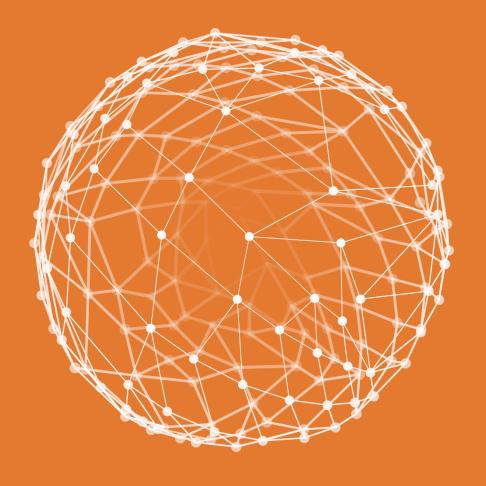


# Remetinostat: next steps

- 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 NDA
- Phase III study will enroll treatment-experienced patients
- Medivir aims to identify a business partner for the further development of remetinostat.



# 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: 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%



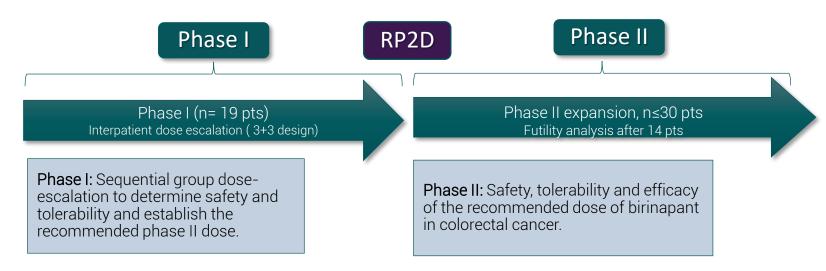
# Birinapant may benefit patients with inadequate response to immuno-oncology therapies

- 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
  - o Keytruda® provided at no cost by Merck
  - Medivir retains full global rights to birinapant and data

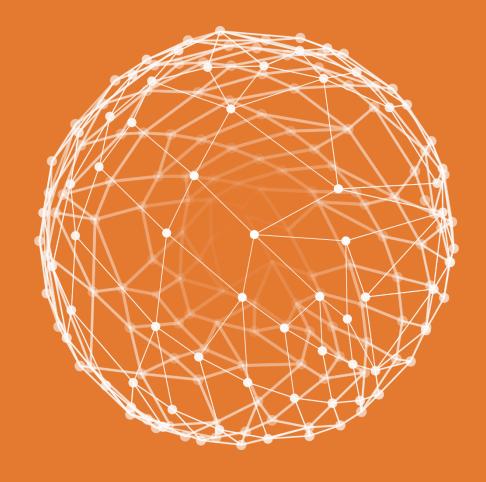


# Birinapant/Keytruda® combination - phase II study ongoing

- Dose escalation completed; December 2018: n=19
  - o One CRC patient has achieved partial response, which had been maintained for over 1 year
  - o Two patients had stable disease for 18 weeks
  - Safety and tolerability: No concerns
  - o Phase II dose selected at 22 mg/m2
  - First patient in phase II study dosed in Dec 2018



# 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
  - o Five-year survival: 18%
  - Genetically heterogeneous leading to limited effect of molecularly targeted therapies
- Intrahepatic cholangiocarcinoma is the second most common primary liver tumor
  - Medium survival is only twelve months
- Existing treatment options provide very little survival benefit

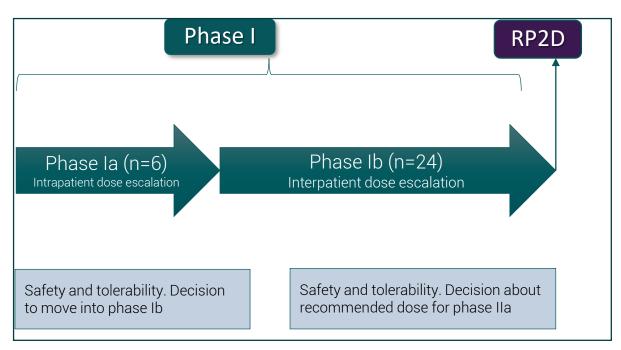


# MIV-818: prodrug for enhanced efficacy and safety in liver cancer (HCC) therapy - Phase I started in Q4 2018

### Troxacitabine

 Clinically active but failed due to systemic dose-limiting toxicities

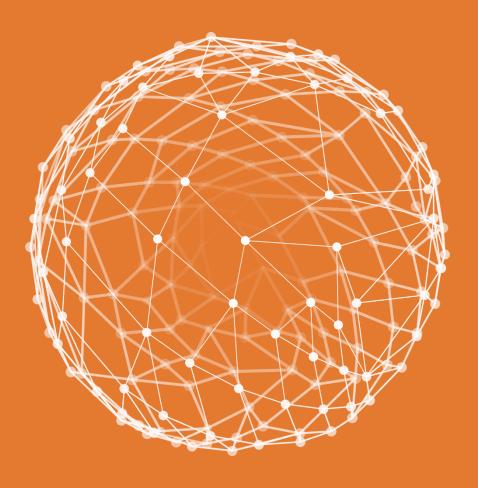




### MIV-818

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

# Corporate information



# **About Medivir**

- Founded in 1988
- Two medicines developed to the market
- Listed on the Nasdaq Stockholm Main Board
- Market cap as of Dec 2, 2019: approximately SEK 600 million
- Cash position as of Sept 30, 2018: SEK 357 million
- Located in Huddinge, Sweden



# **Recent milestones**

- Birinapant/Keytruda®: completion phase I study Q4 2018
- Birinapant/Keytruda®: start of phase II study Q4 2018
- Remetinostat: positive EOP2 meeting with the FDA Q4 2018

# Near term value inflection points

- MIV-818: completion phase la study Q2 2019
- Birinapant/Keytruda®: futility analysis completed Q4 2019

