



INVESTING IN ONCOLOGY
STOCKHOLM, 7 OCTOBER 2019

MEDIVIR

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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	
Remetinostat HDAC INHIBITOR (TOPICAL)	Cutaneous T-cell lymphoma (MF)	Completed					IP: 2034	
	Basal cell carcinoma ¹⁾	Completed		Ongoing			IP: 2034	
Birinapant SMAC MIMETIC (INTRAVENOUS)	Solid tumors (combo with Keytruda®)	Completed			Ongoing		IP: 2034	
MIV-818 NUCLEOTIDE DNA POLYMERASE INHIBITOR (ORAL)	Hepatocellular carcinoma	Completed		Ongoing			IP: 2035	
MIV-828 NUCLEOTIDE BASED DNA POLYMERASE INHIBITOR (INTRAVENOUS)	Blood cancer (acute myeloid leukemia)	Completed	Ongoing					IP: Est 2039
MIV-711 CATHEPSIN K INHIBITOR (ORAL)	Osteoarthritis	Completed					IP: 2034	

¹⁾ Investigator sponsored study at Stanford U.

Completed Ongoing



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

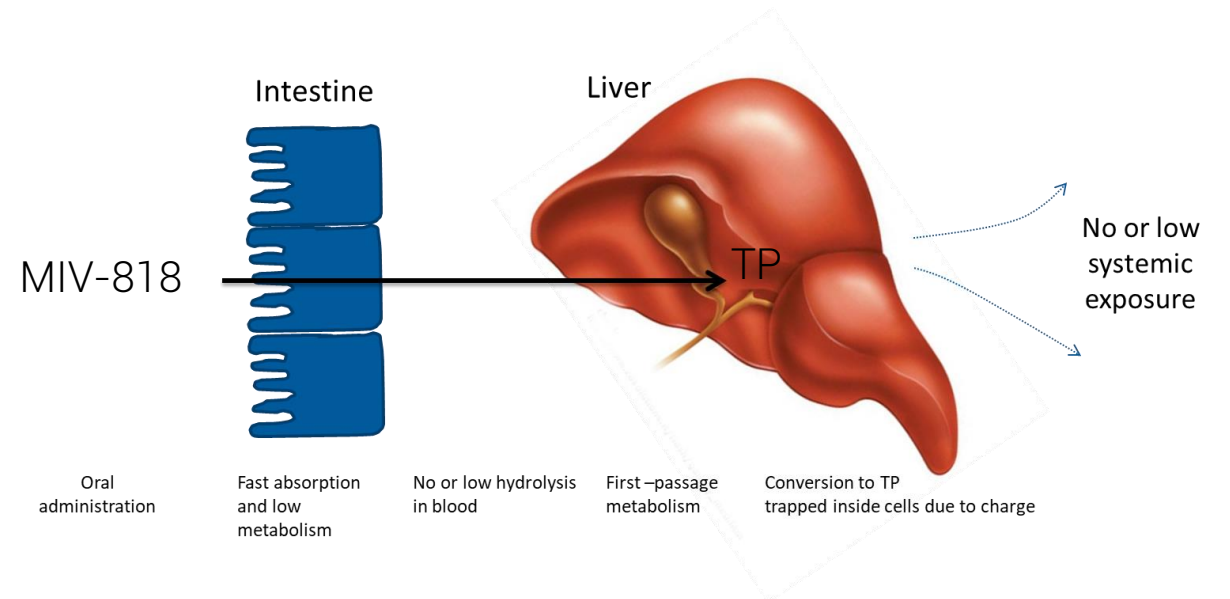
Primary liver cancer: hepatocellular carcinoma and intrahepatic cholangiocarcinoma

- 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: 11% for regional disease and 2% for distant disease
 - Genetically heterogeneous leading to limited effect of molecularly targeted therapies
- Intrahepatic cholangiocarcinoma (iCCA) is the second most common primary liver tumor
 - Median survival is only 12 months
- Existing treatment options provide very little survival benefit with no drugs approved for iCCA

MIV-818 for treatment of liver cancer

Liver targeting

- MIV-818 is a troxacitabine (TRX)-based prodrug, designed to increase generation of the active metabolite TRX triphosphate (TP) in the liver after oral dosing
- When incorporated into DNA, TP causes double strand DNA breaks and cell death



Prodrug approach

- Increase generation of TP in liver by first-pass uptake and rapid intracellular conversion to non-permeable charged metabolites
- Minimize systemic exposure to troxacitabine

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

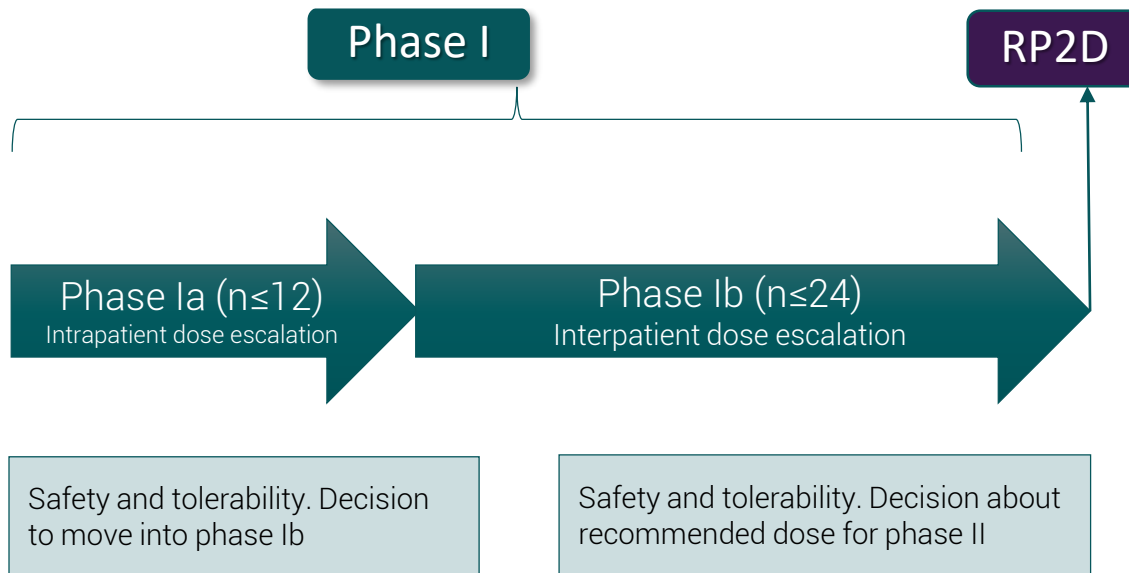
Troxacitabine (iv)

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



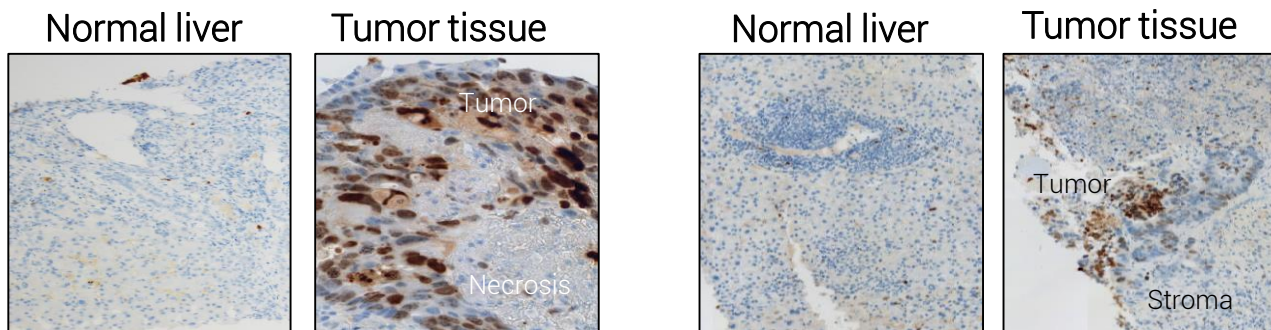
MIV-818 (oral)

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



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

- Data analyzed from six patients with advanced liver cancer treated with escalated doses of MIV-818
- MIV-818 was well tolerated. Lowering of blood counts in the patient at the highest dose suggests that we are close to the maximal tolerated dose
- Clear signs of effect, measured as DNA damage, in biopsies from liver cancer tissue. No DNA damage seen in normal liver tissue
- DNA damage also observed in hypoxic liver cancer regions
- Signs of effect on the size of the liver tumors in several patients
- Tumor selective effect observed at low MIV-818 levels in plasma



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

Milestones

Medivir - recent and upcoming milestones

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