



# MEDIVIR

Improving life for cancer patients  
through transformative drugs

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February 2017

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# Oncology drug development in areas of high unmet need

## Strong and balanced development pipeline...

	Project, Mechanism	Disease area	Preclinical	Clinical phase			Market	Next step
				Phase I	Phase II	Phase III		
Cancer	<b>Remetinostat</b> Topical HDAC inhibitor	<b>Early-stage cutaneous T-cell lymphoma</b>	[Progress bar: Preclinical to Phase II]				~\$1b US only	P3 start 2018
	<b>Birinapant</b> SMAC mimetic	<b>Solid tumors</b> (combo with Keytruda®)	[Progress bar: Preclinical to Phase I]				Blockbuster	P2 start 2H2018
	<b>MIV-818</b> , Nucleotide DNA polymerase inhibitor	<b>Hepatocellular carcinoma</b>	[Progress bar: Preclinical]				Orphan US/EU Significant Asia	P1 start 2H2018
	<b>MIV-711</b> Cathepsin K inhibitor	<b>Osteoarthritis</b>	[Progress bar: Preclinical to Phase II]				Blockbuster	Partner

...leveraging specialist drug discovery expertise...

**Protease inhibitors**  
(example: deubiquitinases)

**Nucleoside prodrugs**  
(example: Leukotide)

■ Protease related  
■ Nucleot(s)ide related

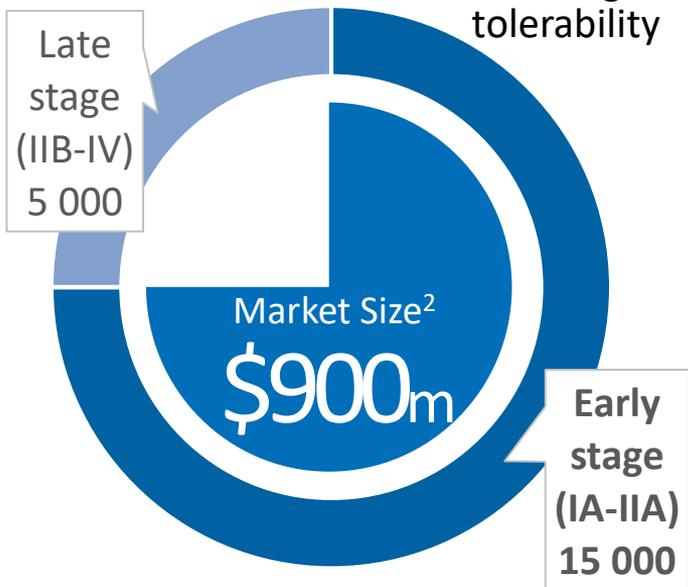
...and key competences

**Cancer biology, chemistry, intellectual property, DMPK, CMC, toxicology, clinical development, regulatory strategy, business development**

## Addresses key unmet need with positive Phase II data

**US CTCL patients<sup>1</sup>:  
orphan disease**

**Key unmet need:**  
balance of efficacy  
and long-term  
tolerability



### Effect on lesions & reduction of pruritus (itch)

Dose	1% 1x/day n=20	0.5% 2x/day n=20	1% 2x/day n=20
Lesion responses <sup>3</sup>	20%	25%	40%
Patients with clinically significant pruritus <sup>4</sup>	8/20 (40%)	6/20 (30%)	10/20 (50%)
Pruritus responses	37.5%	50%	80%

### Highly tolerable with no systemic side effects

- Even dose distribution of AEs, mostly grade 1 or 2
- No HDAC inhibitor-associated systemic adverse events
- Median time on treatment: 332 days (1% 2x/day dose)

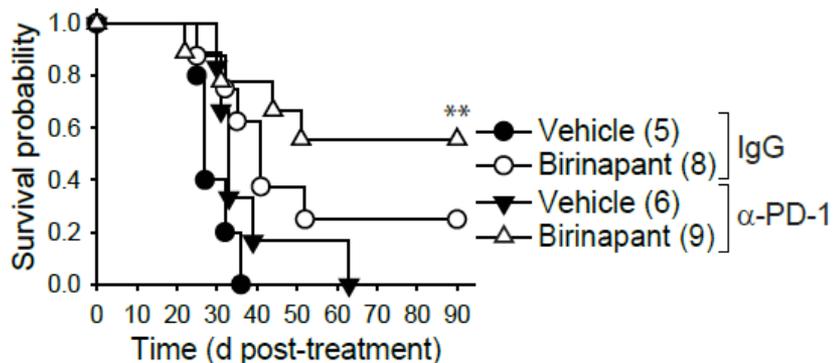
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1( Leukemia & Lymphoma Society; 2) Early-stage patients at \$60 000 per patient year price based on market research and competitive topical treatment pricing. The Medical Letter, Issue 1467, April 27, 2015 and Actelion public information; 3) Confirmed responses based on CAILS, the Composite Assessment of Index Lesion Severity; 4). Clinically significant pruritus defined at baseline as VAS ≥30 mm

# Potential to enhance patient response with immune-oncology therapies

## Strong rationale for combination with Keytruda®

- Birinapant/anti-PD1 mAb combo showed enhanced activity in preclinical models<sup>1</sup> compared to either agent alone



<sup>1</sup> Solid tumor model: Beug et al., Nature Communications (2017) 8:14278  
Multiple myeloma model: Chesi et al., Nature Med. (2016) 22, 1411–1420

## Phase I/II study underway in collaboration with MERCK

- Development collaboration for the Phase I/II study in solid tumors
- Keytruda® provided at no cost
- Joint Development Committee to oversee the study, bringing Merck's immuno-oncology expertise
- Medivir retains full global rights to birinapant and data

# Potential to improve efficacy and safety for patients with liver cancers

## Liver cancer<sup>1</sup>

- Orphan disease in Western markets, but much more common in Asia
- One of fastest growing and most deadly cancers in US
- Genetically heterogeneous leading to limited effect of molecularly targeted therapies

## Improve a nucleoside with Medivir prodrug technology

**Troxacitabine**  
(nucleoside)



**MIV-818**

(liver-targeted nucleotide prodrug)

- **Active** in preclinical cancer models and in clinic
- Failed in clinic due to systemic dose-limiting **toxicities**
- **Enhanced activity** 10x more potent against HCC cell lines than parent troxacitabine
- **Selectivity for cancer** for HCC cells relative to non-cancerous human hepatocytes
- **Improved delivery to the liver** of greater than 100-fold compared to the parent nucleoside

# MIV-711: ORAL ONCE DAILY CATHEPSIN K INHIBITOR BENEFITS BOTH BONE AND CARTILAGE IN OSTEOARTHRITIS

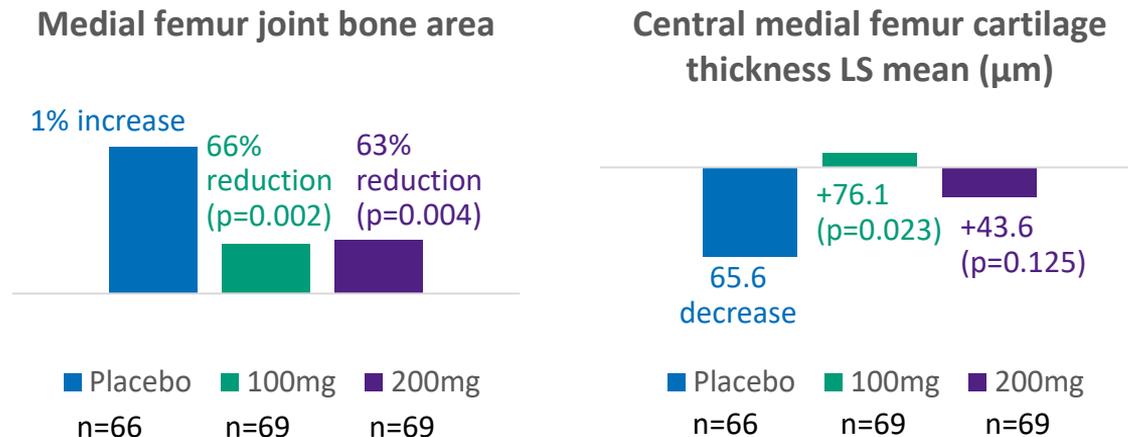
## Phase IIa data show unprecedented OA disease modification after 6 months

### No existing disease modifying drug for Osteoarthritis

- Affects >30m adults in the US, and ~240m worldwide
- Disease involves both bone and cartilage

Annual US market potential<sup>1</sup>  
**>\$6bn**

### Benefit on both bone and cartilage in Phase IIa study



- Positive trends across all pain and other patient reported outcomes
- Acceptable safety and tolerability profile

# Why Medivir?

## Basic facts

- Headquarters in Huddinge, Sweden
- 80 employees, 43 with PhDs
- Listed on the Nasdaq Stockholm, ticker: MVIR
- Website: [www.medivir.com](http://www.medivir.com)

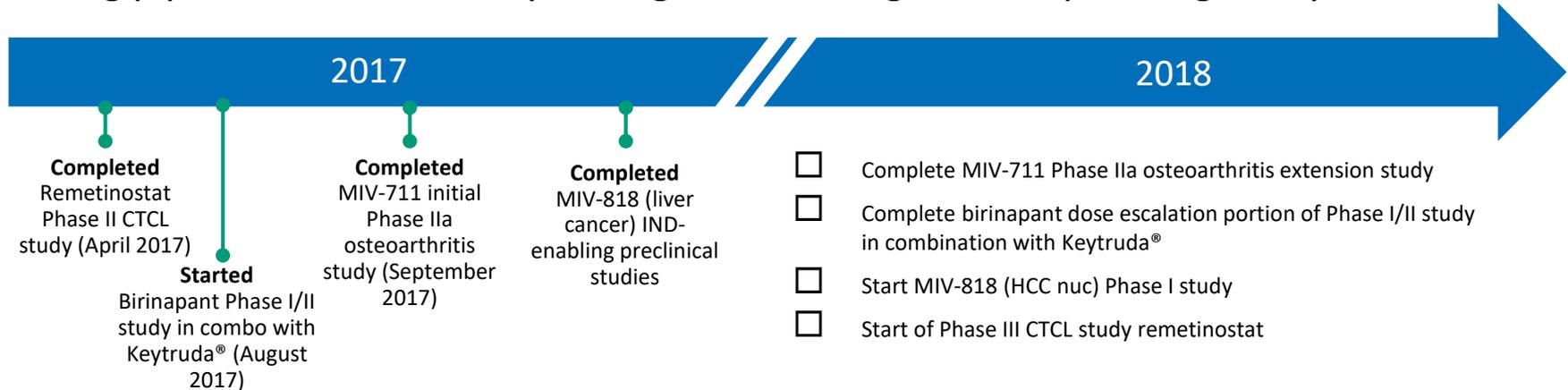
- Track record of delivery

3 new drugs into  
development in 2 years

2 products from  
idea to market

>20 global partnerships,  
multiple repeat partners

- Strong pipeline from discovery through clinical stages with upcoming catalysts



- Near-term opportunity for partnership