

A laboratory setting featuring a robotic arm positioned over two racks of blue microcentrifuge tubes. The tubes are arranged in a grid pattern, and the scene is illuminated with a cool blue light. The background is dark and out of focus, showing more laboratory equipment.

# MEDIVIR

Improving life for cancer patients  
through transformative drugs

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April 2017

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# Improving life for cancer patients through transformative drugs

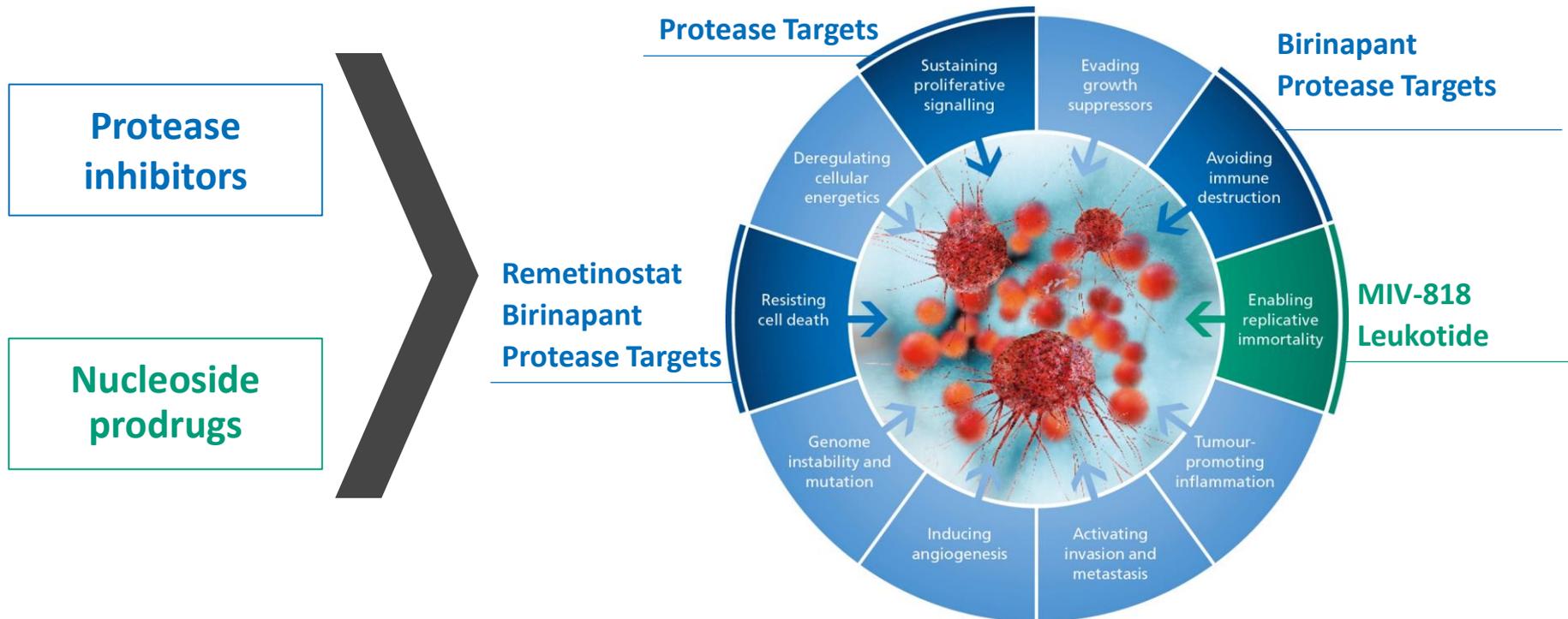
- Using **world-class scientific expertise** to bring new therapies to cancer patients
- **Clinical pipeline** composed of projects with **multi-billion dollar sales** potential as well as **orphan cancer drug candidates**
- Strong commercial focus – delivered more than **20 global partnerships** and **2 products from idea to market**

## Basic facts

- Headquarters in Huddinge, Sweden
- 77 employees, 43 with PhDs
- Listed on the Nasdaq Stockholm, ticker: MVIR
- Current market capitalization: SEK 967m (~USD 125m)<sup>1</sup>
- Website: [www.medivir.com](http://www.medivir.com)



# Leveraging scientific expertise to build pipeline in oncology



Adapted from: The Hallmarks of Cancer: The Next Generation.  
Hanahan and Weinberg, Cell (2011), 144, 646-674

# Oncology drug development in areas of high unmet need

Strong and balanced development pipeline based around areas of scientific expertise and focused on cancer

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

■ Protease related  
■ Nucleot(s)ide related

# Collaborations enhance the value of programs

## Academic



## Industrial

Product/Project

Platform Link

Partners

Status

Medivir Interests

Zoviduo®/Xerclear  
(labial herpes)  
*acyclovir + hydrocortisone*

Nucleoside  
analogue



Marketed

- Royalties from sales
- Approval milestones for additional OTC switches

MIV-802 (HCV)  
*Nucleotide NS5B  
polymerase inhibitor*

Nucleotide



Phase I ready

- Development milestones
- Royalties from sales

# Competences from discovery through regulatory approvals

## Management team with extensive experience and proven track record of successful development



**RICHARD BETHELL**, Chief Scientific Officer

- 28 years drug discovery and development in oncology and infectious disease
- VP, Biology/DMPK (Boehringer Ingelheim (Canada))
- VP, Therapeutic Research (Shire)
- Pfizer and GlaxoSmithKline R&D
- Doctor of Philosophy (D. Phil.) in chemistry from Oxford University



**JOHN ÖHD**, Chief Medical Officer

- Senior director of Experimental Medicine, Shire
- Early development group director, cognitive and neurodegenerative disorders at AstraZeneca
- Cancer research at Lund University and at Karolinska Institute
- Clinical training at Karolinska University Hospital
- MD, Linköping University, PhD in Experimental Pathology, Lund University



**ÅSA HOLMGREN**, EVP Strategic Regulatory Affairs

- Head of Regulatory Affairs at Orexo AB
- Various large pharmaceutical companies, including 12 years as Senior Global Regulatory Affairs Director at AstraZeneca, and at AstraZeneca in Canada and Japan
- M. Sc. in Pharmacy, trained Uppsala University



**CHRISTINE LIND**, President and CEO

- EVP, Business Development at Medivir
- VP, Business Development, LifeCell Corporation
- Biotech and pharma strategic advisory and capital raising at Merrill Lynch & Co. and GKM & Co.
- B. Sc. Finance and Info Systems, NYU and MBA, Columbia Business School



**ERIK BJÖRK**, Chief Financial Officer

- CFO for AstraZeneca Sweden Operations
- 11 years with Procter & Gamble, in global finance leadership positions in Switzerland, UK and Sweden
- MSc in Finance and LLM from Lund University



**CHRISTINA HERDER**, EVP Strategic Business Development

- CEO of Modus Therapeutics
- Director, Corporate Development at Sobi
- Project & Portfolio Management at Biovitrum
- Current member of the boards of PCI Biotech and Idogen
- Ph. D. in physical chemistry from Royal Institute of Technology and MBA from Stockholm University



**DANIEL ERIKSSON**, Chief Information Officer

- Various IT roles relating to security, decision support, innovation, and digitalization
- Most recently, Technical Director for G4S Risk Management
- PhD Coventry University and BSc in Systems Science, Linköping University

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

77 employees, 43 with PhDs,  
18 nationalities, balanced gender split

# MIV-711: ORAL ONCE DAILY CATHEPSIN K INHIBITOR WITH FDA FAST TRACK STATUS FOR OA DISEASE MODIFICATION

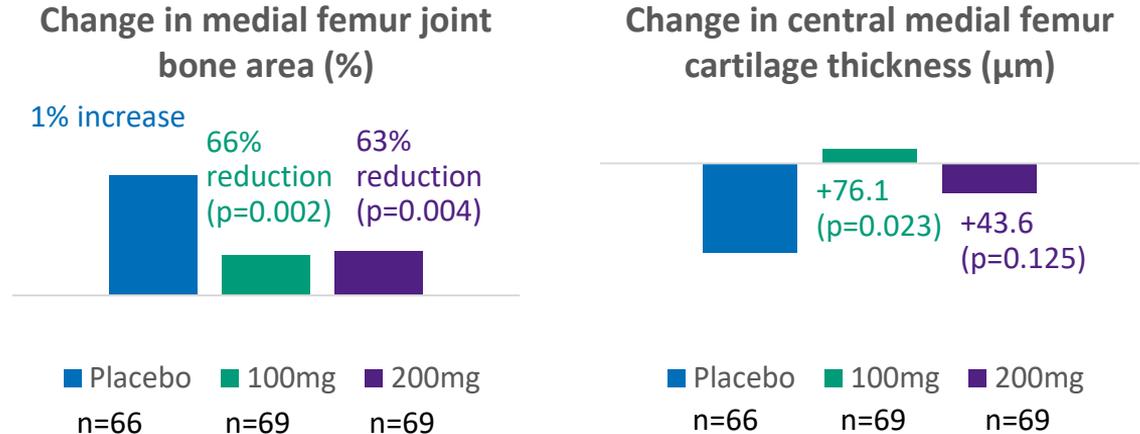
## Phase IIa data show unprecedented OA disease modification after 6 months

### No existing disease modifying drug for Osteoarthritis

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



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



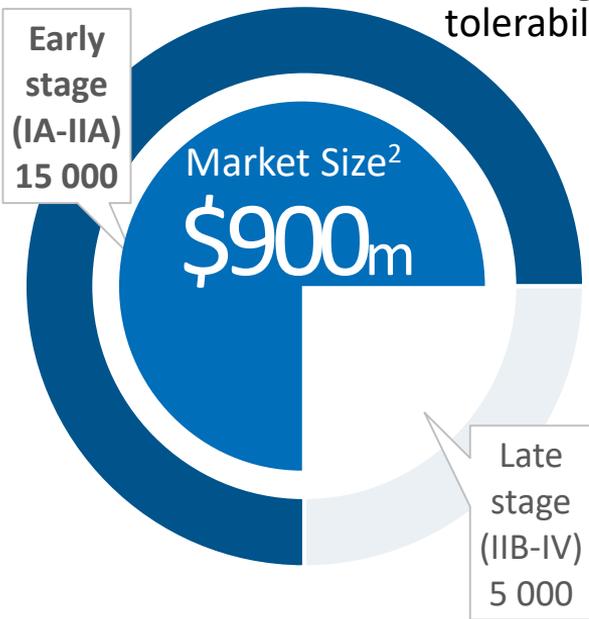
### Acceptable safety and tolerability profile

- Both doses showed acceptable safety and tolerability for this patient population

## 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%

M Duvic *et al.*, EORTC Cutaneous Lymphoma Task Force Meeting (2017), Abstract O55

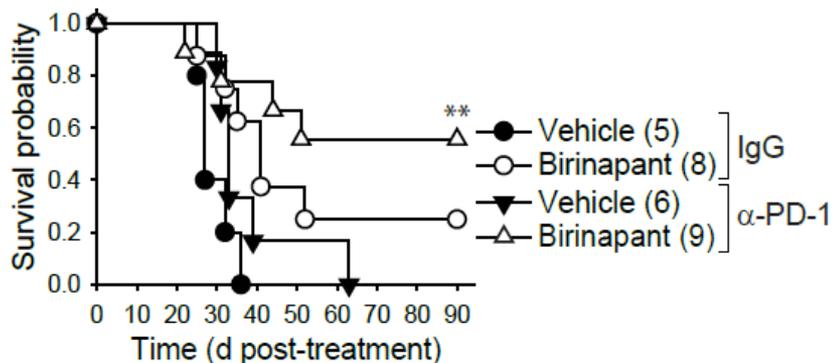
### Highly tolerable with no systemic side effects

- 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)

# 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  
Cooperation of IAPs and PD-L1 to protect tumor cells: Kearney et al., Cell Death and Diff. (2017), 24, 1705–1716

## Phase I/II study underway in collaboration with



- 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
- **Delivery to the liver improved** by greater than 100-fold relative to systemic exposure of troxacitabine itself

# Cash position and shareholder base

## CASH POSITION

Cash and ST investments  
SEK468m (USD 60m)



Directed offering: SEK155m  
(USD 20m) gross proceeds

Successful out-licensing:  
additional cash mid-term

Investments in R&D  
Estimated 18+ months  
cash runway

End 2017

## THE SHARE

Market Cap<sup>1)</sup>  
~967m SEK  
(~USD 125m)

International  
Ownership  
~31%

Top 20  
shareholders  
~51%

# Why Medivir?

**For more information:**

- Nasdaq Stockholm, ticker: MVIR
- [www.medivir.com](http://www.medivir.com)

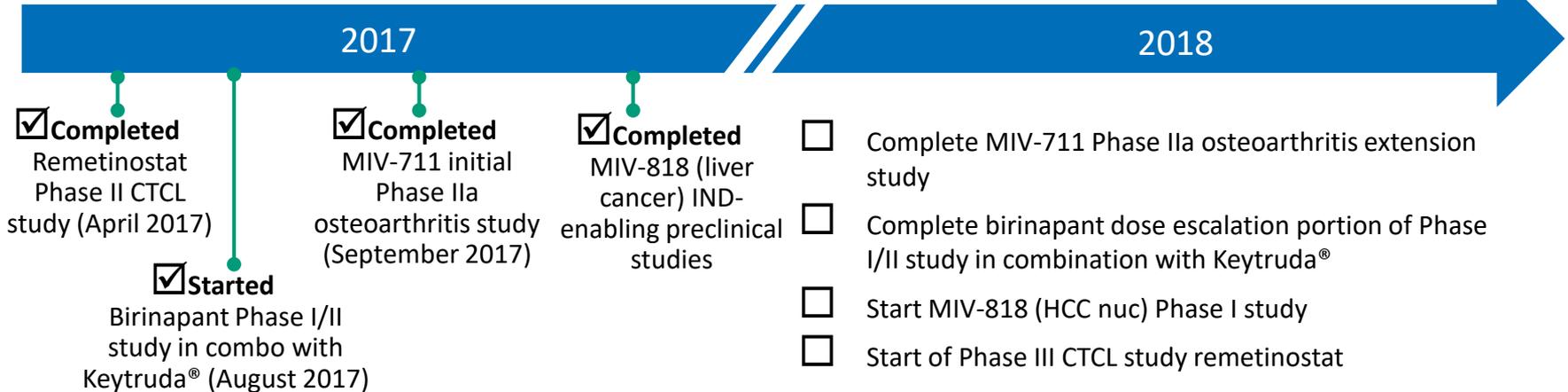
- Track record of delivery

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 partnerships