MEDIVIR

Improving life for cancer patients through transformative drugs

Stockholm Corporate Finance Life Science Seminarium

March 2018

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Medivir in Brief

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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 790m (USD 100m)[1]
→ Website: www.medivir.com

1) As of February 16, 2018. Stock price 38,90 SEK

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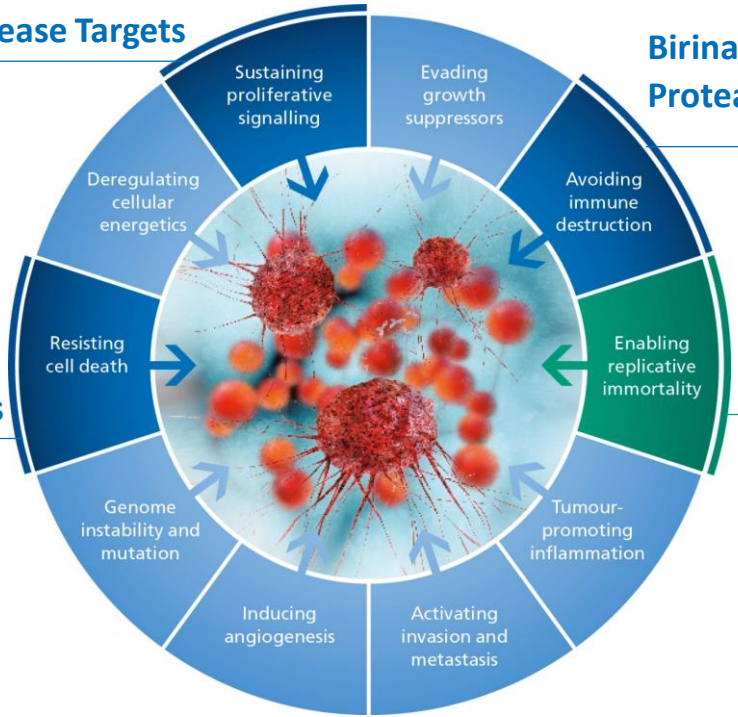
Leveraging scientific expertise to build pipeline in oncology

Protease inhibitors

Nucleoside prodrugs

Protease Targets

Remetinostat
Birinapant
Protease Targets



Birinapant
Protease Targets

MIV-818
Leukotide

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

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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	**Remetinostat** Topical HDAC inhibitor	**Early-stage cutaneous T-cell lymphoma**		~$1b US only	P3 start 2018
	Birinapant SMAC mimetic	**Solid tumors** (combo with Keytruda®)		Blockbuster	P2 start 2H2018
	MIV-818, Nucleotide DNA polymerase inhibitor	**Hepatocellular carcinoma**		Orphan US/EU Significant Asia	P1 start 2H2018
	MIV-711 Cathepsin K inhibitor	**Osteoarthritis**		Blockbuster	Partner

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Partner

Collaborations enhance the value of programs

Academic

The University of Nottingham
UHN Princess Margaret Cancer Centre
Stanford Health Care
University of Leeds
Karolinska Institutet
UT Southwestern Medical Center
Uppsala Universitet
Stockholm University
Newcastle University
UCLA
University of Leicester

Industrial

Product/Project	Platform Link	Partners	Status	Medivir Interests
Zoviduo®/Xerclear (labial herpes) *acyclovir + hydrocortisone*	Nucleoside analogue	gsk	Marketed	▪ Royalties from sales ▪ Approval milestones for additional OTC switches
MIV-802 (HCV) *Nucleotide NS5B polymerase inhibitor*	Nucleotide	TREK Therapeutics 歌礼中国 Ascletis	Phase I ready	▪ Development milestones ▪ Royalties from sales

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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 Astra Zeneca
- 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

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MIV-711 for Osteoarthritis

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No existing disease-modifying drug for osteoarthritis

Blockbuster revenue opportunity for a disease-modifying osteoarthritis drug

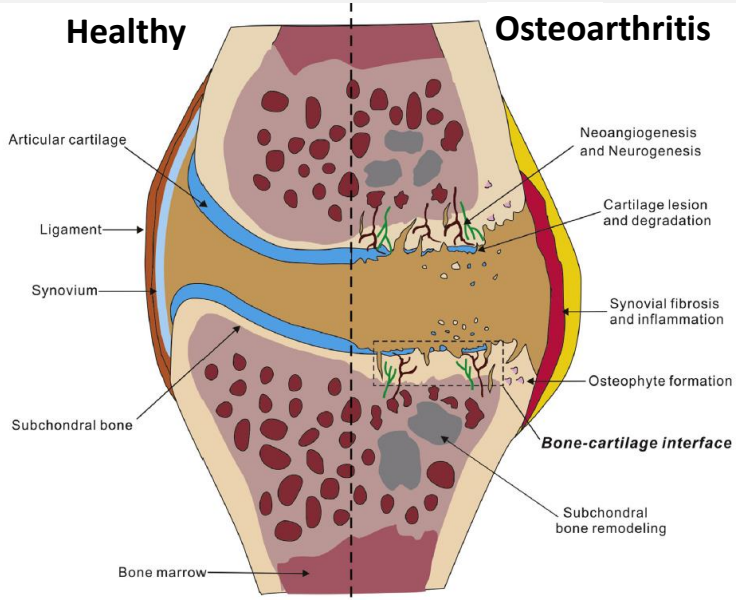
- Affects >30m adults in the US, and ~240m worldwide
- Prevalence increasing due to aging population and obesity epidemic
- Current treatments are insufficient focusing only on symptom relief

Annual US market potential[1]

>$6bn

Disease involves both bone and cartilage

- Cathepsin K protease involved in the breakdown of collagen in both bone and cartilage



Yuan *et al.* Osteoarthritis and Cartilage (2014), 22, 1077

Sources: Hunter et al, Nat Rev Rheumatol, 2014; Reginster et al, Ann Rheum Dis 2013.
1) >2M adults in US with moderate osteoarthritis in weight bearing joints at annual treatment cost for a drug that impacts disease progression of 3,000 USD/Year (Losina et al 2014)

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Phase IIa data show unprecedented OA disease modification after 6 months

Potential disease-modifying convenient osteoarthritis drug

- Only cathepsin K-inhibitor in development for osteoarthritis
- Once daily oral administration

Strong patent position

- Expected patent life to ~2034, including extensions

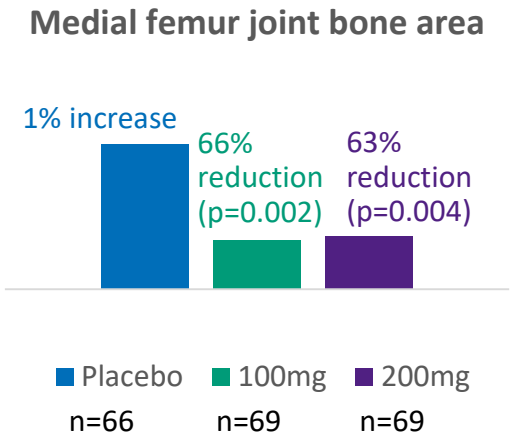
US FDA Fast Track designation

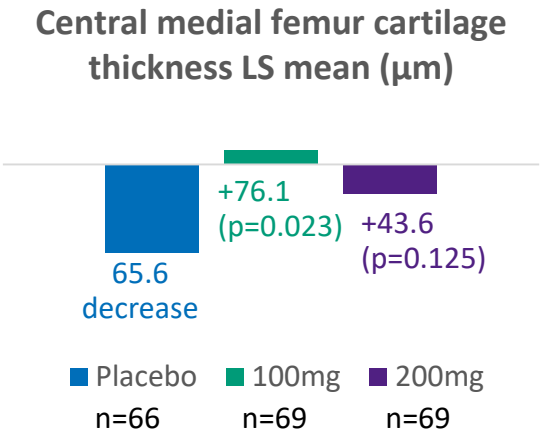
- Granted by FDA October 2017

"The finding that MIV-711 slows the degenerative changes on both bone and cartilage in knees affected by OA is an enormously exciting finding"

Professor Philip Conaghan, Professor of Musculoskeletal Medicine at the University of Leeds, UK, and lead investigator on the MIV-711-201 study

Benefit on both bone and cartilage in Phase IIa study





- MIV-711 consistent tendency to improve outcomes across all pain and other patient reported symptoms vs. placebo
- Acceptable safety and tolerability profile

http://acrabstracts.org/ Abstract 14L

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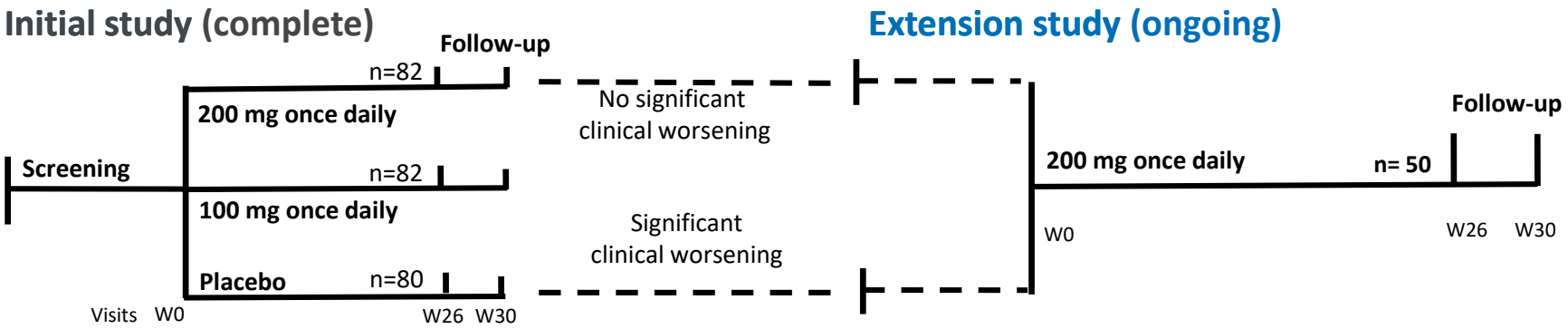
MIV-711: Ongoing development and future plans

- Partnering discussions ongoing
- Additional 12 and 6 month efficacy data from extension study expected 1H'18



Remetinostat for early-stage CTCL

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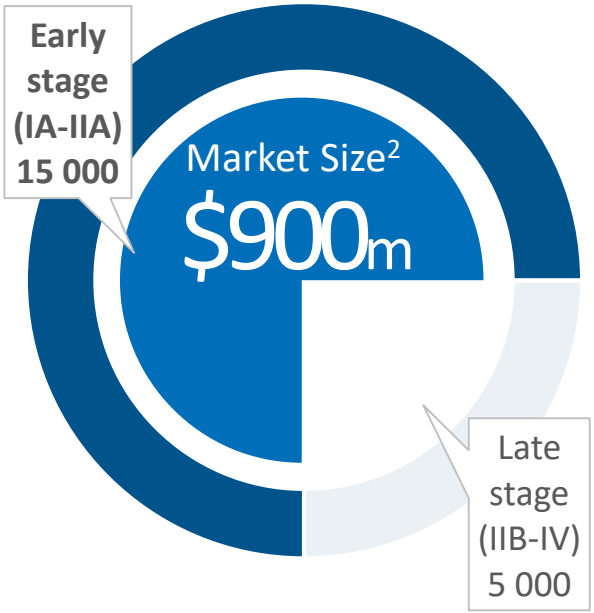
CTCL: orphan blood cancer with significant market opportunity

US CTCL patients[1]: orphan disease



Early Stage CTCL: Disease background

- Confined to the skin
- High 5-year survival rates (~85%)
- Patients remain at this stage for extended periods and require long-term treatment
- Significant quality of life issues, especially pruritus (itch)

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

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

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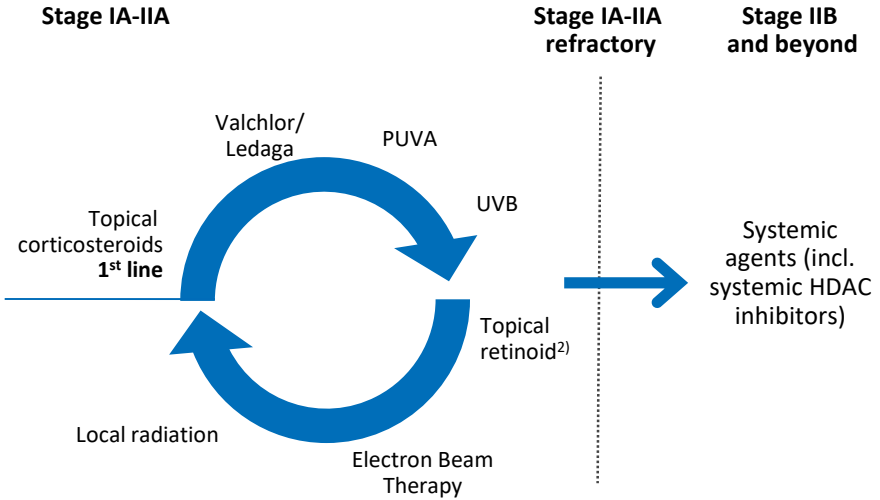
Patients & physicians need new treatments for early-stage CTCL

- Currently approved early-stage CTCL drugs lack sustained efficacy and/or tolerability and are highly irritating
- Valchlor/Ledaga is a topical alkylating agent (the active agent in mustard gas)
- No single treatment for long-term use
- At this early stage, physicians avoid using systemic drugs, including other HDAC inhibitors, due to the side effect profile

Currently approved therapies by disease stage



1) Medivir market research; 2) Full approval in USA only

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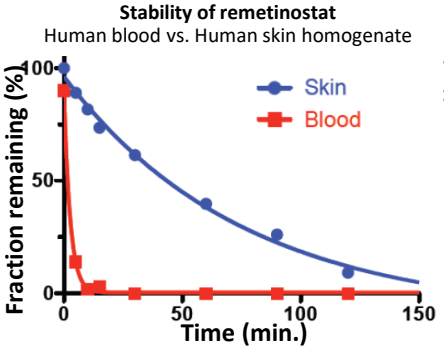
Remetinostat potential to meet patients' key unmet need

Designed to act only where needed

- HDAC inhibitors[1] approved in late-stage CTCL patients but not in early-stage patients due to systemic toxicities
- Remetinostat's unique design and topical application provides activity in skin, but rapid degradation in blood



- Expected patent life to ~2034 (including extensions)
- US orphan drug designation

"As a topical, skin-specific HDAC inhibitor, remetinostat has the potential to be efficacious and have an improved safety profile compared to other available treatments."

Youn Kim M.D.
Stanford University Medical Center, USA

1) HDAC inhibitor = histone deacetylase inhibitor

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Addresses key unmet need with positive Phase II data

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[1]	20%	25%	**40%**
Patients with clinically significant pruritus[2]	*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)

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

1) Confirmed responses based on CAILS, the Composite Assessment of Index Lesion Severity;
2) Clinically significant pruritus defined at baseline as VAS ≥30 mm

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Planned Phase III clinical development for early-stage CTCL

Design

One Phase III study expected to be sufficient for NDA

- Past approvals in CTCL were based on pivotal clinical studies involving <260 patients
- Focus on treatment-experienced patients where the medical need is high

Program Timing

- Phase II final data reported (EORTC CLTF Meeting, 2017)
- Ongoing discussions with the FDA (End of Phase II) to enable Phase III

Costs

- ~$50m (SEK 400m) expected clinical costs to NDA submission over a ~3 year period (incl. Phase III study and third party milestones)

"The introduction of remetinostat to the market as a novel topical agent for the treatment of CTCL is likely to find broad application and lead to novel combinatorial approaches in CTCL."

Pierluigi Porcu, M.D.
Jefferson University Hospital , USA

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Birinapant for solid tumors

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Despite immuno-oncology breakthroughs patients have unmet needs

Multi-billion dollar market, and growing for immuno-oncology agents

Revenues of PD-1 inhibitors [1)]

$8bn

< 1/2

of patients derive meaningful clinical benefit in approved indications

0%

ORR in other indications such as MSS colorectal cancer

Combination regimens to enhance benefit in underserved patients

1) Merck (Keytruda) and Bristol-Myers Squibb (Opdivo) financial reports, twelve months ended September 30 2017

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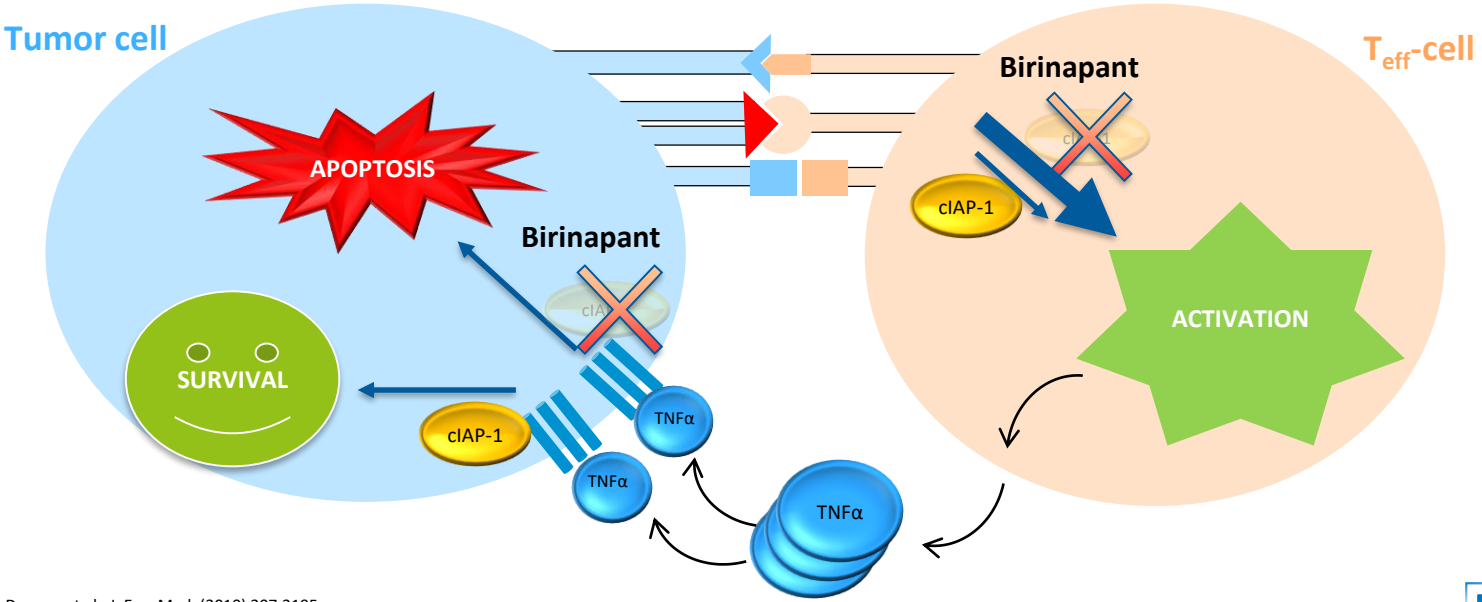
Dual action enhances cancer cell death

- Enhances pro-apoptotic, and suppresses pro-survival, signaling downstream of TNF-α
- Augments human T cell responses to physiologically relevant stimuli[1]



1) Dougan et al., J. Exp. Med. (2010) 207:2195

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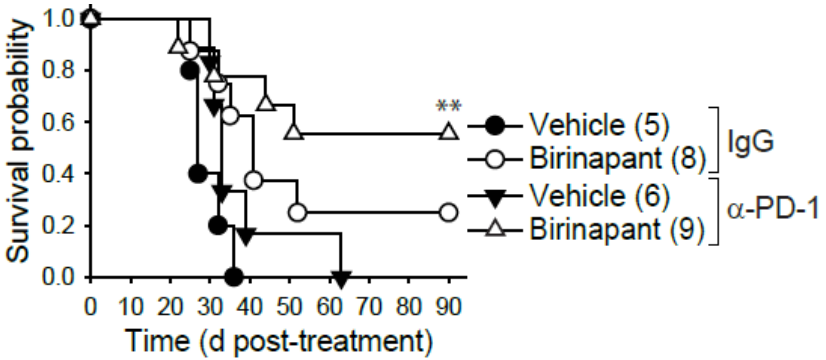
Potential to enhance patient response with immune-oncology therapies

Strong rationale for combination with Keytruda®

- Birinapant/anti-PD1 mAb combo showed enhanced activity in preclinical models[1] compared to either agent alone



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

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Birinapant/Keytruda® combination: Phase I/II Study underway

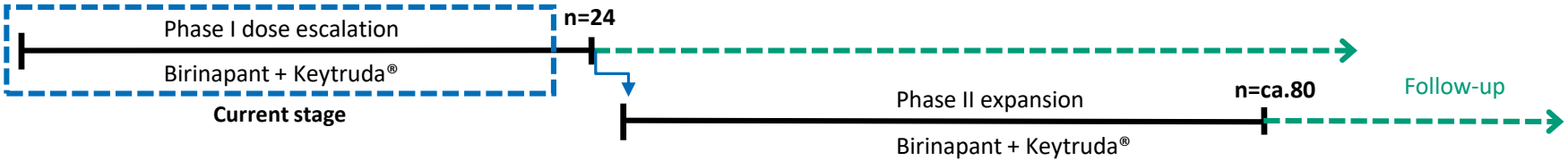
Collaboration with MERCK

- 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

Costs

- ~$20m (SEK 160m) expected costs to completion of planned studies (incl. Phase I/II study over 3 years; no third party milestones)

Design



Phase I: Sequential group dose-escalation to determine the dose-limiting toxicity and recommended Phase II dose, in combination with Keytruda®

Phase II: Safety and tolerability of the recommended dose of birinapant, in combination with Keytruda® in 3-4 defined disease cohorts

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MIV-818 for liver cancers

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Liver cancer is 2nd leading cause of cancer related death worldwide

Liver cancer[1]

- 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

Patients with advanced liver cancer in need of new treatments

Sorafenib

(kinase inhibitor)

- ~3 month survival benefit

→

Regorafinib

(kinase inhibitor)

- ~3 month survival benefit

Nivolumab

(PD-1 antagonist)

- 15-20% ORR
- Survival benefit yet to be fully defined

1) Howlader et al. (eds). SEER Cancer Statistics Review, 1975-2011, National Cancer Institute. Bethesda, MD, http://seer.cancer.gov/csr/1975_2011/

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Potential to improve efficacy and safety for patients with liver cancers

Improve a nucleoside with Medivir prodrug technology

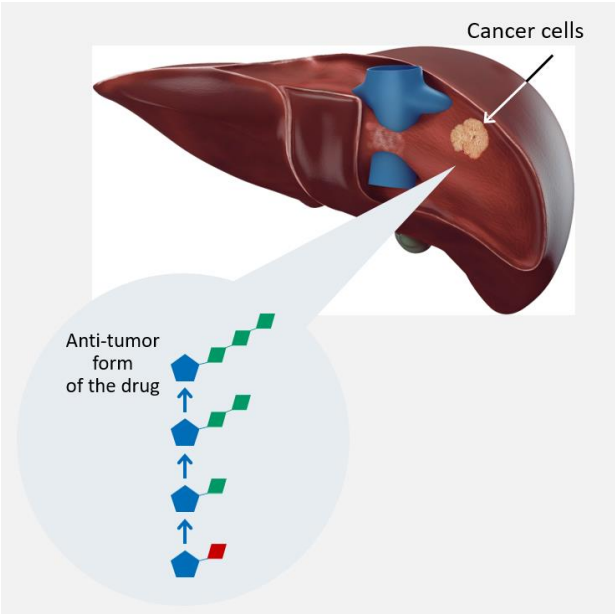
Troxacitabine
(nucleoside)

- **Active** in preclinical cancer models and in clinic
- Failed in clinic due to systemic dose-limiting **toxicities**

→ Medivir prodrug technology →

MIV-818
(liver-targeted nucleotide prodrug)

- **Exhanced activity** 10x more potent against HCC cell lines than parent troxacitabine
- **Selectivity for cancer** Active on HCC cells while sparing non-cancerous hepatocytes
- **Improved delivery to the liver** >10-fold increased delivery of the active drug to the liver, with a 10-fold reduction in exposure to troxacitabine elsewhere



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MIV-818: Ongoing development and future plans

Significant interest in MIV-818

- GLP safety studies completed in January 2018
- Documentation being prepared for submission to regulatory authorities
- Phase I study planned to start in second half of 2018

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Outlook

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Cash position and shareholder base

CASH POSITION

Cash and ST investments SEK468m (USD 60m)

End 2017

+

Directed offering: SEK155m gross proceeds

Successful out-licensing: additional cash mid-term

Investments in R&D Estimated 18+ months cash runway

THE SHARE

Market Cap[1)] ~790m SEK (USD 100m)

International Ownership ~36% of capital

Top 20 shareholders ~57% of votes

1) As of February 16, 2018. Stock price 38,90 SEK

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Why Medivir?

Basic facts
- Headquarters in Huddinge, Sweden
- 77 employees, 43 with PhDs
- Listed on the Nasdaq Stockholm, ticker: MVIR
- Current market capital: SEK 790m (USD 100m)[1]
- Website: www.medivir.com

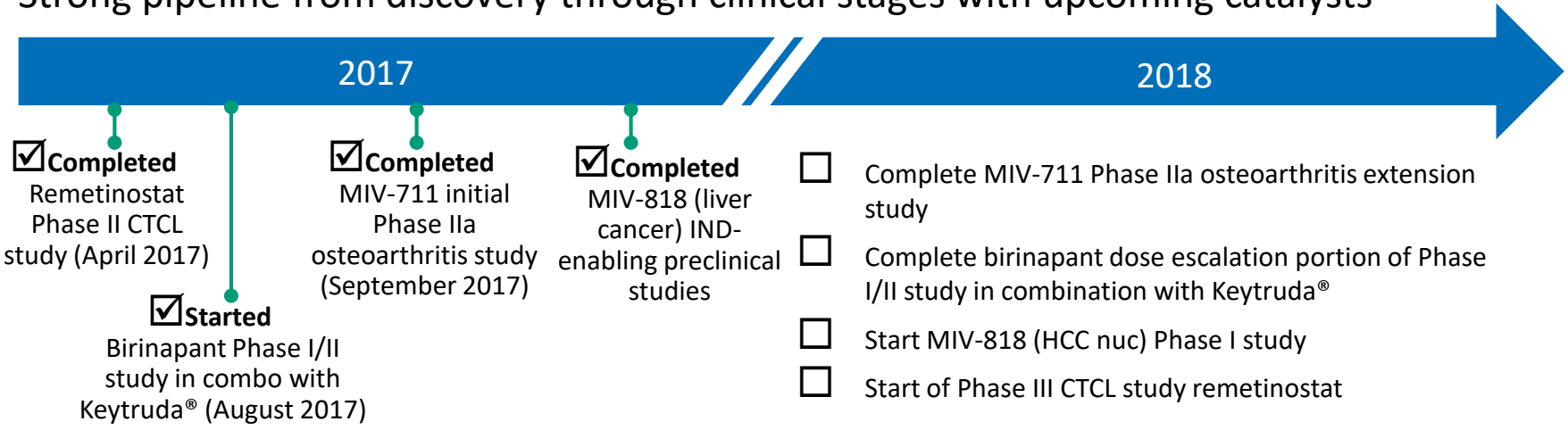
- Track record of delivery

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

1) As of February 16, 2018. Stock price 38,90 SEK

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