

A laboratory setting with a pipette and two microplates containing small vials. The scene is lit with a blue glow.

MEDIVIR

Third Quarter

October 26, 2017

Third Quarter Highlights

Positive data reported across multiple clinical programs

- Positive topline results from phase IIa osteoarthritis study showed disease-modifying benefit and an acceptable safety and tolerability in patients
- Remetinostat phase II data demonstrated efficacy on skin lesions, reduction of itching and high tolerability in patients with early-stage CTCL
- Clinical study of birinapant in combination with KEYTRUDA® in patients with treatment-refractory solid tumours was initiated

Total royalties revenues of 5.1 MSEK in Q3

Publication date for the Financial Statement 2017 moved from 16 February 2018 to 14 February 2018

In order to more rapidly advance the development of our clinical pipeline, Medivir has appointed Carnegie Investment Bank, as lead financial adviser, with Kempen & Co N.V. to evaluate the company's capital structure and possible funding options

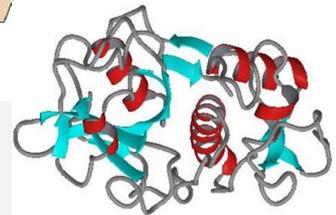
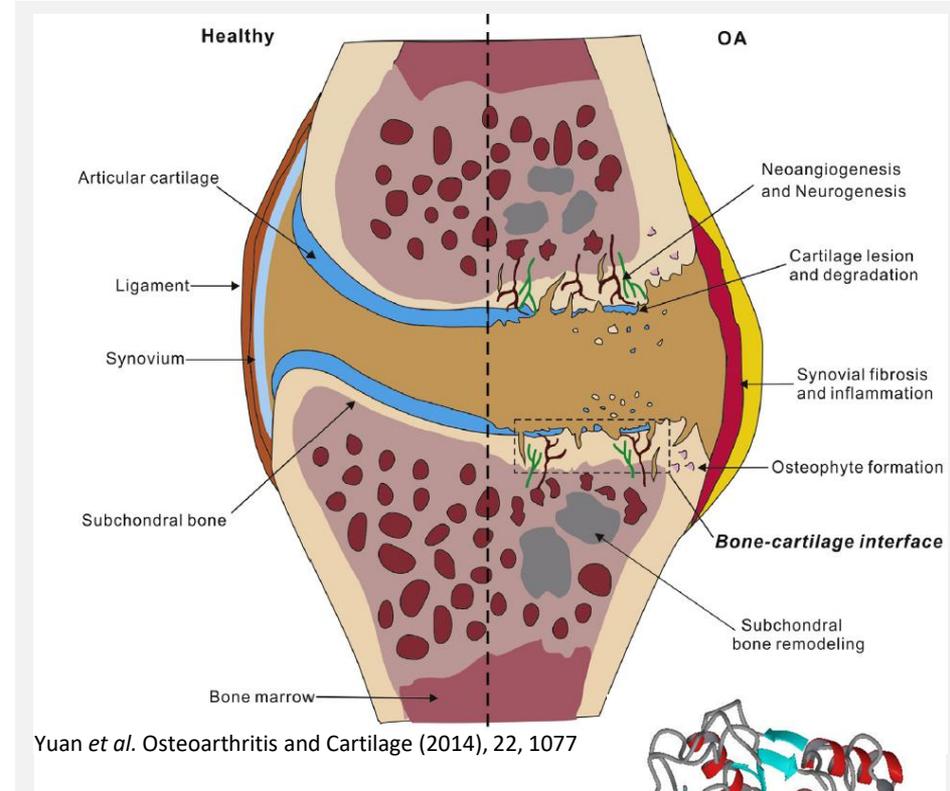




Program Highlights

MIV-711: potential for first disease-modifying drug in osteoarthritis

- Most advanced disease-modifying drug in development for osteoarthritis
- Blockbuster revenue potential
- Good market exclusivity with expected patent life to ~2034, including extensions
- Phase IIa data demonstrated unprecedented joint structure benefit in very short time



No disease-modifying osteoarthritis drug exists today

- Osteoarthritis is a disease involving both bone and cartilage
- Prevalence increasing due to aging population and obesity epidemic
- Current treatments are insufficient focusing on symptom relief only



Blockbuster revenue opportunity for a disease-modifying OA drug (DMOAD)

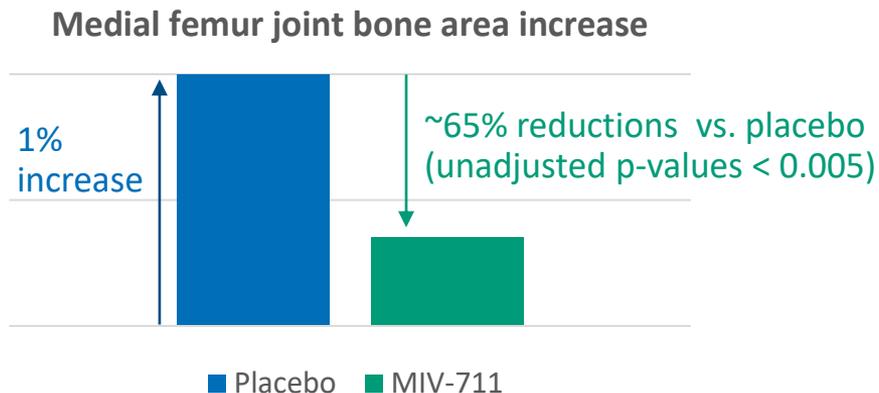
Sources: Hunter et al, Nat Rev Rheumatol, 2014; Reginster et al, Ann Rheum Dis 2013

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)

Phase IIa data showed unprecedented benefit on joint structure in 6 months

Benefit on bone

- Increasing joint bone area is a key marker of osteoarthritis and disease progression
- Both MIV-711 doses substantially reduced medial femur bone area growth relative to placebo¹:



Benefit on cartilage

- MIV-711 reduced loss of cartilage thickness on the medial femur versus placebo (unadjusted $p=0.023$ for the 100mg dose, 0.125 for the 200mg dose)¹

Acceptable safety and tolerability profile

- Both doses showed acceptable safety and tolerability for this patient population
- Six independent DMC meetings held during the Phase IIa program have reviewed unblinded safety data and concluded “continue as planned”

Continuing to advance MIV-711 development

- Phase IIa 201 study data accepted for late-breaking presentation at the 2017 Annual Meeting of the American College of Rheumatology (November 3-8)
- Fast track designation granted by FDA (October 24)
- Partnering discussions initiated
- Additional 12 and 6 month efficacy data from extension study expected 1H'18

“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

Remetinostat: proven mechanism with unique positioning

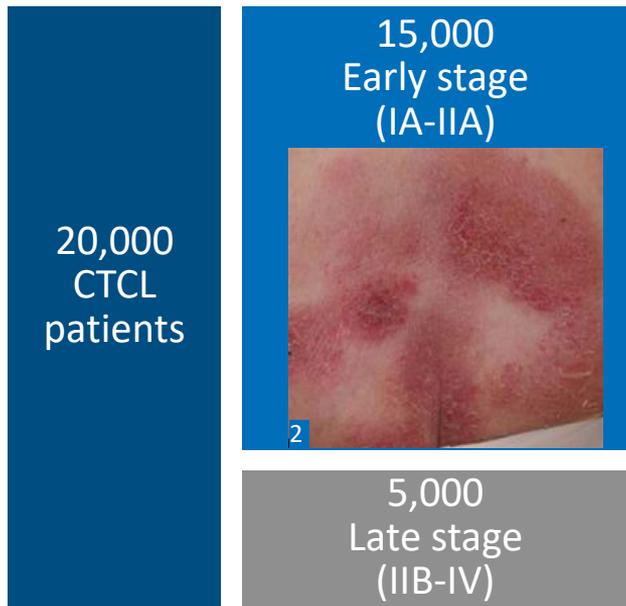
- Proven mechanism in late-stage cutaneous T-cell lymphoma uniquely positioned as a topical treatment with early-stage patients
- Consistent annual revenue potential
- Good market exclusivity with expected patent life to ~2034 and US orphan drug designation
- Positive phase II safety & efficacy data, presented at EORTC Cutaneous Lymphoma meeting (October 15)
- Opportunity for expansion into other indications

"In short, 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 combination approaches in CTCL."

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

CTCL: orphan blood cancer with significant market opportunity

US CTCL patient demographics¹



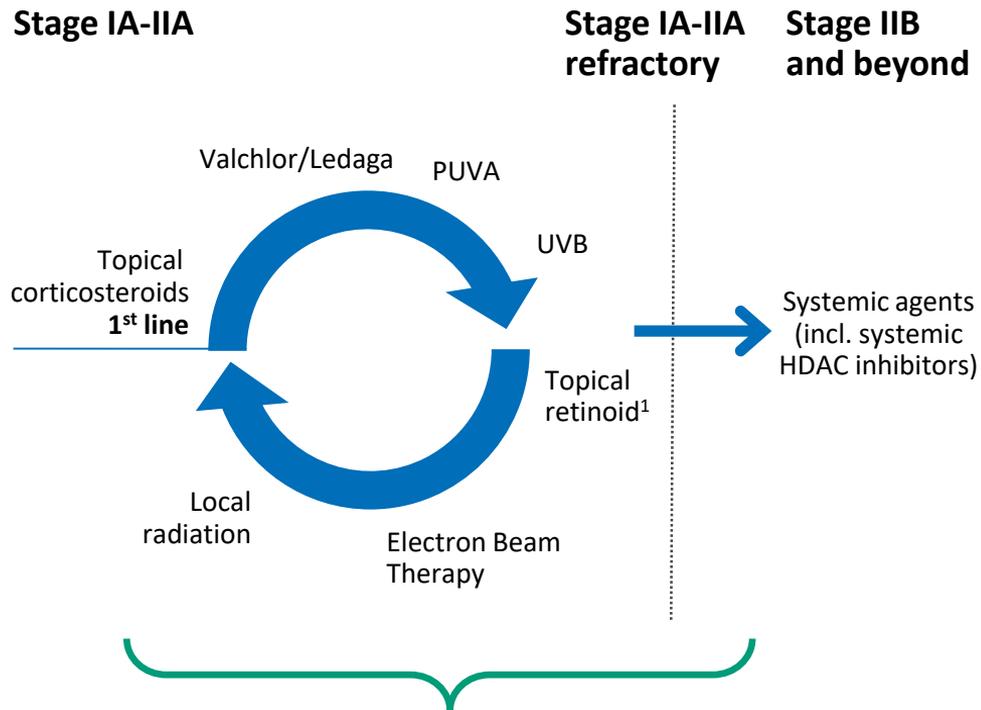
- Orphan disease
- Cancerous T-cells confined to the skin
- Patients remain at this stage for extended periods and require long-term treatment
- Significant quality of life issues, especially pruritus (itch)

>\$60K
per patient year pricing³

Early-stage CTCL market⁴
\$900m

Patients & physicians need new treatments for early-stage CTCL

- Currently approved drugs lack sustained efficacy and/or tolerability and are highly irritating
- No single treatment for long-term use
- Physicians avoid using systemic drugs due to side effect profile



Phase II data – good effect on skin lesions and reduction of pruritis (itch)

	1% 1x/day n=20	0.5% 2x/day n=20	1% 2x/day n=20
<i>Lesion Outcomes</i>			
CAILS¹ Confirmed Overall Response Rate (% of patients)	20%	25%	40%
Median Duration of CAILS Confirmed Response²	2 months	3 months	7 months
<i>Pruritus Outcome</i>			
<i>Patients with clinically significant pruritus at baseline (VAS ≥30 mm at baseline)</i>	<i>8/20 (40%)</i>	<i>6/20 (30%)</i>	<i>10/20 (50%)</i>
Confirmed response in patients with clinically significant pruritus at baseline (% of patients)	37.5%	50%	80%

1. Composite Assessment of Index Lesion Severity; 2. Estimated from visit dates, censored at study end.

Phase II safety data – highly tolerable with no systemic adverse events

- Tolerable profile, mostly grade 1-2 and no worse for highest dose
- No HDAC inhibitor-associated systemic adverse events
- Median time on treatment: 332 days (1% 2x/day dose)

Treatment-related AEs seen in ≥1 Patient ¹	No. of Patients (n=20/group)		
	1% 1x/day	0.5% 2x/day	1% 2x/day
Any AE	11	10	11
Pruritus	5	3	1
Any Other Skin²: irritation, dermatitis, erythema, dry skin, rash, exfoliation, skin lesion, inflammation, pain, paraesthesia, erythema, application site reaction	9	10	11
Infections	3	1	0
Skin papilloma	0	0	1

1. Patients are counted once in each system organ class category; 2. Excludes pruritus

Planned phase III clinical development for early-stage CTCL

Design

1% 2x/day selected for phase III:

- Dose response observed on CAILS & pruritus
- Balanced AE profile across all three doses

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, in whom medical need is high

Program Timing

- End of Phase II meeting with FDA to enable Phase III (4Q 2017)
- Phase III start 2018
- Potential for launch in 2022

Costs

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



“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

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



Financial Summary

Financial Summary

Summary of Group's figures (SEK m)	Q3		Q1-Q3		Full Year
	2017	2016	2017	2016	2016
Net turnover *)	5.1	25.7	32.4	83.2	93.0
EBITDA	-78.3	-53.9	-250.1	-174.9	-300.6
Basic earnings per share	-3.94	-1.87	-11.42	-4.85	-10.50
Diluted earnings per share	-3.94	-1.87	-11.42	-4.85	-10.47
Cash flow from operating activities	-63.6	-37.0	-269.6	-110.5	-182.3
Liquid assets and ST investments	557.9	955.0	557.9	955.0	1 698.5

*) Net turnover in Q3 totalled SEK 5.1 (25.7) MSEK, of which SEK 5.1 (13.5) MSEK comprised third quarter royalties for simeprevir and Xerclear

Improving life for cancer patients through transformative drugs

Strong development pipeline...

...leveraging specialist drug discovery expertise...

Nucleoside prodrugs
(primary focus:
targeted delivery)

Protease inhibitors
for cancer
(primary focus:
deubiquitinases)



Project, Mechanism	Disease area	Preclinical phase		Clinical phase		
		Discovery	Preclinical	Phase I	Phase II	Phase III
Remetinostat Topical HDAC inhibitor	Early-stage cutaneous T-cell lymphoma	[Progress bar: Discovery to Phase II]				
	Basal cell carcinoma (Stanford investigator sponsored study)	[Progress bar: Discovery to Phase I]				
Birinapant SMAC mimetic	Solid tumors (combo with Keytruda®)	[Progress bar: Discovery to Phase I]				
	High-grade serous carcinomas	[Progress bar: Discovery to Phase I]				
MIV-818 , Nucleotide DNA polymerase inhibitor	Hepatocellular carcinoma	[Progress bar: Discovery to Preclinical]				

...with synergies across the portfolio created by key competences

Cancer biology, chemistry, DMPK, CMC, toxicology, clinical development, regulatory affairs, intellectual property



Q&A

Improving life for cancer patients through transformative drugs

www.medivir.com

Ticker: MVIR

Exchange: Nasdaq Stockholm

For more information please contact
Anders Lundin, interim CFO
(anders.lundin@medivir.com)