

A laboratory setting with a pipette and two racks of microcentrifuge tubes. The scene is dimly lit with a blue glow from the racks. The pipette is positioned above the racks, and the tubes are arranged in neat rows.

# MEDIVIR

## First Quarter

April 28, 2017

# First Quarter Highlights

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## Transformation to R&D focus

- Redemption program implemented and completed
- Outlicensed Olysio and any future simeprevir-containing products in the Nordics to Janssen for royalties and additional commercial milestones
- Commercial rights to Adasuve in the Nordic region returned to Ferrer
- Christine Lind appointed CEO

## Continued progress in R&D proprietary pipeline

- MIV-711 osteoarthritis study final DMC safety review with successful outcome to continue as planned

## Total revenues of 17.8 MSEK in Q1

- Global net sales of Olysio of USD 22.8m, generating royalties of 13.7 MSEK



# Significant events after the first quarter

- New CEO, Christine Lind, effective April 1

## **Additional pipeline advances, including from partners**

- Reported positive phase II topline efficacy data for remetinostat in early-stage CTCL
- Phase IIa data presented at EASL by partner J&J on JNJ-4178 (HCV combination including simeprevir) reiterated and extended previously presented efficacy and safety data





# R&D Update

# CTCL is an orphan blood cancer that affects the skin

## 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)



Patient with CTCL: plaques and patches

*J Clin Aesthetic Dermatol.* 2009;2(6):22–27

Significant quality  
of life issues for  
patients with CTCL

# Patients & physicians need new treatments for early-stage CTCL

## Limitations of current treatments

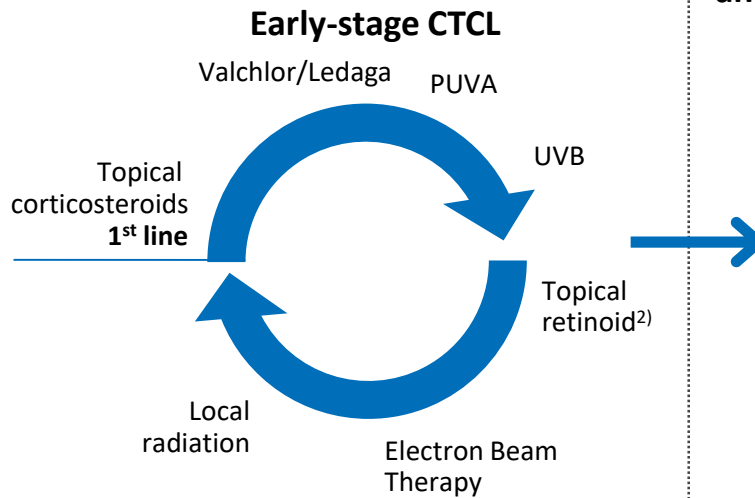
- Currently approved drugs lack sustained efficacy and/or tolerability and are highly irritating
- No single treatment for long-term use
- Available therapies typically used in rotation

## Key unmet needs <sup>1)</sup>

- Tolerability
- Efficacy on non-responding lesions
- Reduction of clinically significant pruritus (itch)

Stage IA-IIA

Stage IIB  
and beyond



**"All the agents currently available for topical use in CTCL have significant side effects, due to skin irritation, and hypersensitivity." Pierluigi Porcu, MD, Jefferson**

<sup>1)</sup> Medivir market research; <sup>2)</sup> Treatments with full approval in USA only

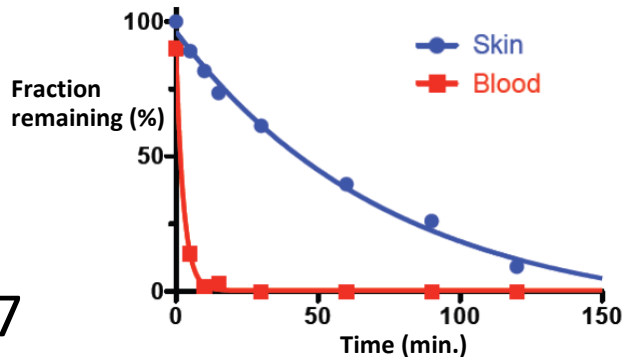
# Remetinostat: Positive Phase II efficacy and safety data in early-stage CTCL

## Remetinostat designed to achieve better efficacy and tolerability balance

- Approved systemic HDAC inhibitors NOT used in early-stage CTCL
  - Effective on disease, but have significant adverse events
- Reteminostat is a topical HDAC inhibitor
- Designed to be effective but decrease toxicity
  - Stable in skin, but degraded rapidly in blood

### Stability of reteminostat

Human blood vs. Human Skin Homogenate



## Positive phase II data in treatment-experienced patients

### Efficacy

Dose	1% QD	0.5% BID	1% BID
CAILS* confirmed responses	4/20 (20%)	5/20 (25%)	8/20 (40%)

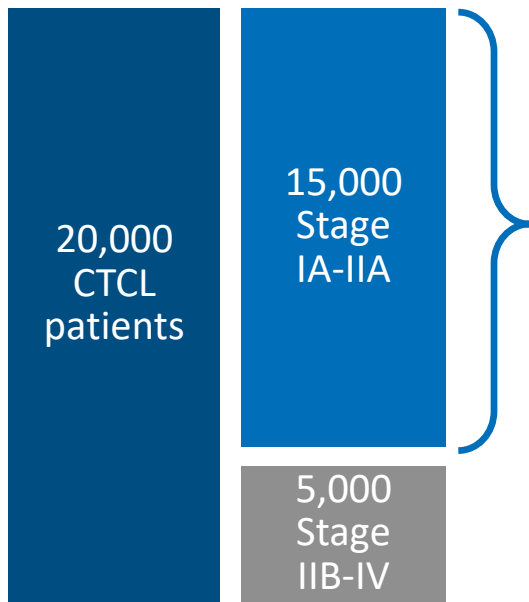
### Safety

- Highly tolerable
  - No adverse events typically associated with systemic HDAC inhibitors were observed

\*Composite Assessment of Index Lesion Severity

# CTCL: orphan cancer disease with significant market opportunity

US patients<sup>1)</sup>



**>\$50k**  
per patient year pricing<sup>2)</sup>



**A 15% market share in the US would translate into over 1 BSEK in annual revenue**

<sup>1)</sup> Leukemia & Lymphoma Society

<sup>2)</sup> Competitive treatment pricing. The Medical Letter, Issue 1467, April 27, 2015 and Actelion public information

<sup>3)</sup> Early-stage patients at expected per patient year price



# Manageable phase III clinical development for CTCL

## About remetinostat

- HDACs: group of enzymes related to proteases
- Topical HDAC inhibitor

### Market Exclusivity

- Expected patent life to around 2034, including extensions
- Remetinostat has orphan drug designation

## Program Timing

- Phase II final data reported April 2017
- End of Phase II meeting with FDA
- Phase III start expected 2H 2017
- Potential for launch in 2021

## Costs

SEK 405m (\$47m) 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, MD, Stanford, California US*

## Ongoing phase IIa studies in osteoarthritis

### Phase IIa progressing as expected

- MIV-711.201 enrollment completed (n=244) end October 2016
- Safety: Final planned MIV-711.201 DMC meeting concluded “continue as planned”
- Phase IIa extension study (MIV-711.202) also on track

### About MIV-711

- Cathepsin K (a protease) inhibitor
- Market exclusivity: expected patent life to around 2034, including extensions

### Timing

- Primary 6 month data expected 3Q'17
- Additional 12 and 6 month data expected 1Q'18

### Costs

~SEK 65m (\$7.4m) expected costs to completion of ongoing Phase IIa studies



**Medivir expects to partner MIV-711 upon successful Phase IIa data**

JNJ-4178

# Phase II data shows JNJ-4178's potential for shortening HCV treatment

**JNJ-4178** *AL-335 + odalasvir + simeprevir* 

Interim PIIa data showed 100% SVR12 in patients receiving treatment for as short as six weeks with the triple combination

Cohort #	Simeprevir dose (mg)	Odalasvir dose (mg)	AL-335 dose (mg)	Treatment duration (weeks)	Number (%) with SVR12 or SVR24
1	100 QD	50 QD	400 QD	8	20/20 (100%), SVR24
2	--	50 QOD	800 QD	8	18/20 (90%), SVR12
3	75 QD	50 QOD	800 QD	8	20/20 (100%), SVR12
4	75 QD	50 QOD	800 QD	6	20/20 (100%), SVR12

QD: every day  
QOD: every other day  
SVR: sustained virologic response

Further information on the trial planning and conduct can be found on [clinicaltrials.gov](https://clinicaltrials.gov) with identifier NCT02765490.

## Status and upcoming milestones

- Phase IIb in non-cirrhotic subjects with HCV fully recruited
  - Efficacy, safety and pharmacokinetics of QD JNJ-4178
  - Hepatitis C virus genotype 1, 2, 4, 5, and 6 infection
  - Six or eight weeks treatment
- JNJ-4178 no longer being developed for genotype 3 infection
- Ongoing phase II study in cirrhotic patients
- Filing for approval expected 2019

## Medivir interests

- Milestones and royalties, if approved



# Financial Summary

# Financial Summary

Summary of Group's figures (SEK m)	Q1		Full Year
	2017	2016	2016
Net turnover	17.8	20.6	93.0
EBITDA	-80.9	-60.7	-300.6
Operation profit (EBIT)	-85.6	-63.7	-312.4
Profit/loss before tax	-84.3	-62.9	-307.7
Basic & Diliuted earnings per share	-3.59	-1.50	-10.50
Net worth per share	38.93	52.39	64.38
Cash flow from operating activites	-123.9	-36.8	-180.1
Liquid assets and ST investments	708.9	1 039.5	1 698,5

- Net turnover totalled SEK 17.8m (20.6m), of which SEK 13.7m (18.1m) comprised first quarter royalties for simeprevir.
- Personnel costs of non recurring nature impacted the total costs negatively by 10.0m (0)

# Result of voluntary share redemption program

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- The redemption program comprised a total of 6,738,655 shares and a total of 6,647,060 shares was registered for redemption whereof;
  - 131,589 series A shares and,
  - 6,515,471 series B shares
- An acceptance level of 98.6 per cent
- Cash proceeds of approximately SEK 857.5 million was distributed to the shareholders
- The total number of outstanding shares in Medivir now amount to 20,318,977 shares whereof;
  - 474,769 series A shares and,
  - 19,844,208 series B shares
- The total number of votes amounts to 24,591,898 votes



# Deep pipeline with multiple value drivers

## Proprietary Pipeline

Diversified from early to late stages of development

Project, Mechanism	Disease area	Preclinical phase		Clinical phase			Market
		Discovery	Preclinical	Phase I	Phase II	Phase III	
<b>Remetinostat</b> Topical HDAC inhibitor	Cutaneous T-cell lymphoma	[Progress bar from Discovery to Phase II]					
<b>MIV-711</b> Cathepsin K inhibitor	Osteoarthritis	[Progress bar from Discovery to Phase I]					
<b>Birinapant</b> SMAC mimetic	Solid tumors*	[Progress bar from Discovery to Phase I]					
	High-grade serous carcinomas	[Progress bar from Discovery to Phase I]					
<b>MIV-818</b> Nucleotide DNA polymerase inhibitor	Hepatocellular carcinoma	[Progress bar from Discovery to Preclinical]					
<b>MIV-323</b> Fusion protein inhibitor	RSV-infection	[Progress bar from Discovery to Preclinical]					

\* Combo with Keytruda™

## Partnership Pipeline

Partnerships where they meaningfully enhance project value

Project	Disease area	Partner	Preclinical phase		Clinical phase			Market
			Discovery	Preclinical	Phase I	Phase II	Phase III	
<b>Olysio (simeprevir)</b>	Hepatitis C	Janssen	[Progress bar from Discovery to Market]					
<b>JNJ-4178</b> AL-335+odalasvir+simeprevir	Hepatitis C	Janssen	[Progress bar from Discovery to Phase II]					
<b>Xerclear</b>	Labial herpes	GSK and Meda	[Progress bar from Discovery to Market]					
<b>MIV-802</b> , nucleotide NS5B polymerase inhibitor	Hepatitis C	Trek Therapeutics	[Progress bar from Discovery to Phase I]					

# Q&A

*Improving life for cancer patients through transformative drugs*



[www.medivir.com](http://www.medivir.com)

**Ticker: MVIR**

**Exchange: Nasdaq Stockholm**

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