

A background image of a laboratory setting with various glassware, including beakers and flasks, on a metal tray. The image is slightly blurred and has a light blue tint.

# Medivir

*A collaborative and agile pharmaceutical company with an R&D focus on infectious diseases and a leading position in hepatitis C*

**Öresundsdagen**

**Lund den 16 September 2013**

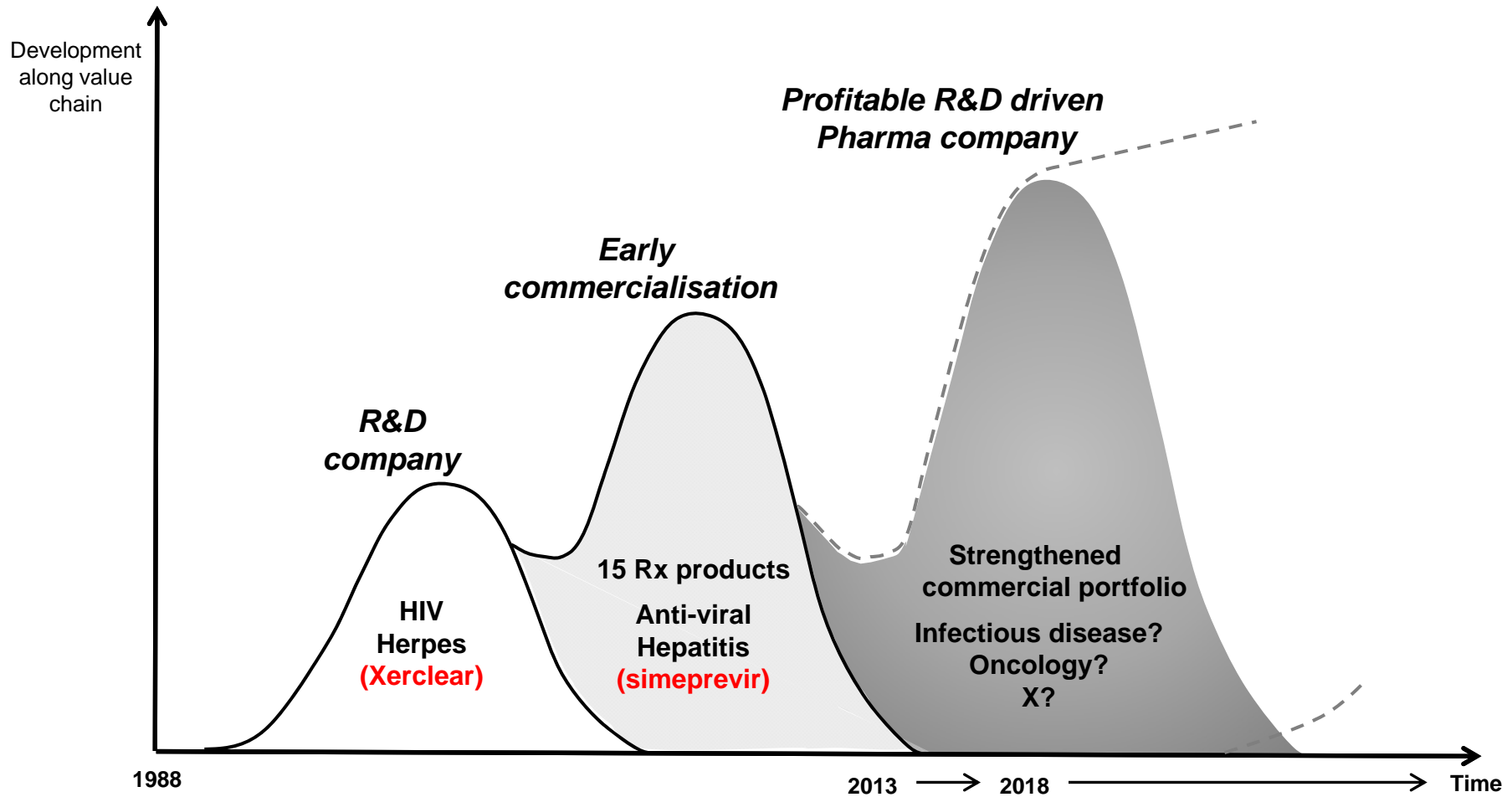
Rein Piir, EVP Corporate Affairs & IR

## Medivir - the emerging European pharma company

- **Integrated pharma company with 15 marketed Rx pharmaceuticals in the Nordics** - annual sales of ~170 MSEK with an EBITDA of ~75 MSEK
- **First in-house developed pharmaceutical (Xerclear) on the market** - the second is approaching market (Simeprevir)
- **Strong position in HCV drug development. Simeprevir, in partnership with Janssen, is considered best in class protease inhibitor** - filed in Japan, the US and Europe in H1, 2013
- **World leading expertise in polymerase and protease drug targets**
- **Extensive partnership track record with major global pharma companies**
- **Solid financial position (400 MSEK on July 1)**



# Transformation of Medivir

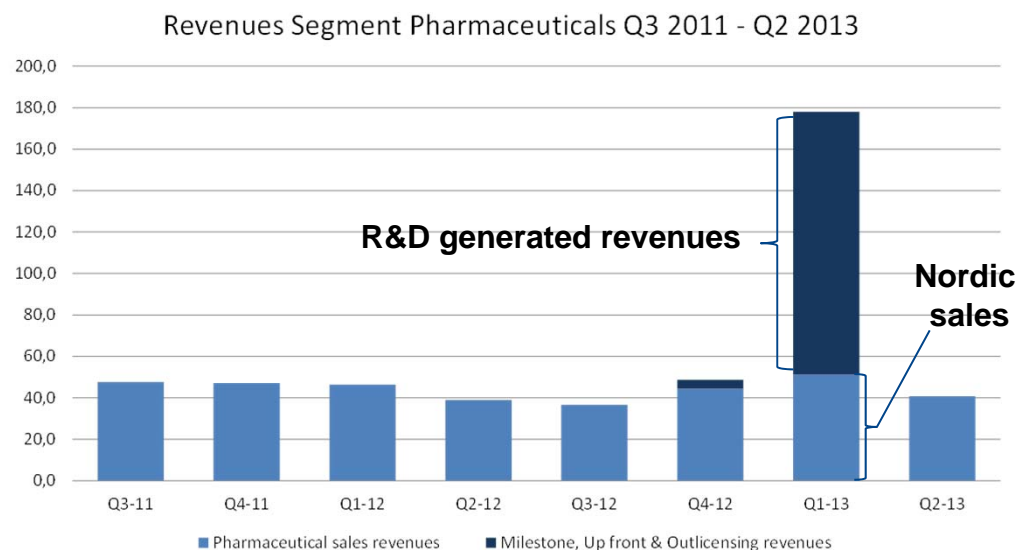


We are on a journey to transform Medivir into a pharma company with long-term sustainable profits

# P&L and quarterly pharmaceutical sales

(SEK m)	2013 Jan-June	2012 Jan-June	2012 Jan-Dec
Net turnover	218.8	85.2	170.6
Gross profit	183.8	54.1	109.3
EBITDA	43.6	-79.6	-165.3
EBIT	14.7	-99	-201.3
Profit/loss before tax	14.5	-98.4	-210.8
Profit/loss after tax	7.5	-107.2	-234.1

Annual net burn rate is ~200 MSEK, excluding milestone and royalty payments



# R&D pipeline status

Field	Project	Partner	Preclinical phase		Clinical phase				Market	
			Re-search	Deve-lopment	Phase I	Phase IIa	Phase IIb	Phase III		
<b>Anivirals</b>										
Labial herpes	Xerclear (Zovido, Zovirax Duo)	GlaxoSmithKline (GSK)								
Hepatitis C	Simeprevir (TMC435), NS3 protease inhibitor	Janssen Pharmaceuticals								
Hepatitis C	NS5B nucleotide-based polymerase inhibitor	Janssen Pharmaceuticals								
Hepatitis C	NS5B nucleotide-based polymerase inhibitor	Unpartnered								
HIV	Protease inhibitor	Janssen Pharmaceuticals								
<b>Other indications</b>										
Bone related disorders	Cathepsin K inhibitor	Unpartnered								
Neuropathic pain	Cathepsin S inhibitor	Unpartnered								





## **Simeprevir**

- A potent HCV protease inhibitor in registration phase**

# Simeprevir - phase III development program in HCV G1 & 4 infected patients

- **QUEST 1 and 2** (treatment-naïve) - *final data presented at EASL*
- **PROMISE** (prior relapser) - *final data presented at Digestive Week*
- **CONCERTO 1-4 in Japan** (treatment naïve & experienced) - *results presented at Japan Society of Hepatology's Annual Meeting*

## *Ongoing phase III studies:*

- **China:** naïve GT1 HCV patients – *fully enrolled (n=444)*
- **ATTAIN:** prior non-responders (SMV vs TVR) – *fully enrolled (n=765)*
- **RESTORE:** HCV GT4 infected patients – *fully enrolled (n=107)*
- **C212: HIV-HCV** co-infected patients – *fully enrolled (n=109)*
- **12 weeks full stop**, open-label, single-arm study in treatment naïve GT1 patients – recruitment ongoing

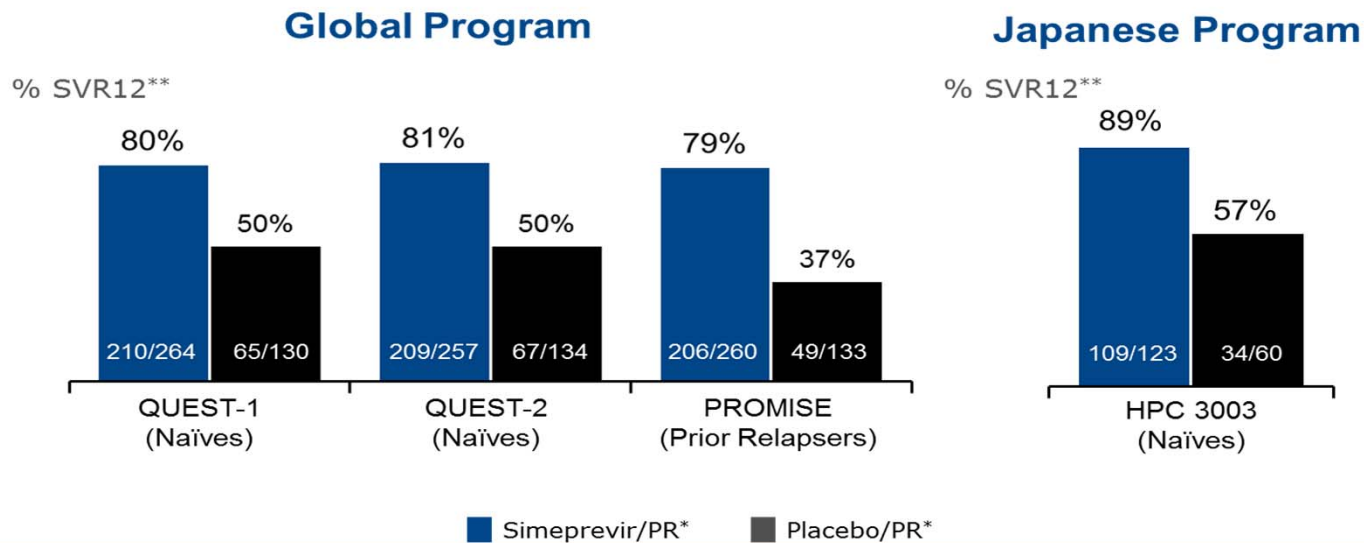
# Simeprevir - Regulatory status and summary phase III

## Regulatory applications filed in:

- **Japan** for hepatitis C genotype 1, naïve, prior non-responders or relapsed – February, 2013
- **US** for hepatitis C genotype 1 – Priority Review granted in May, 2013
- **EU** for hepatitis C genotype 1 and 4 – April, 2013

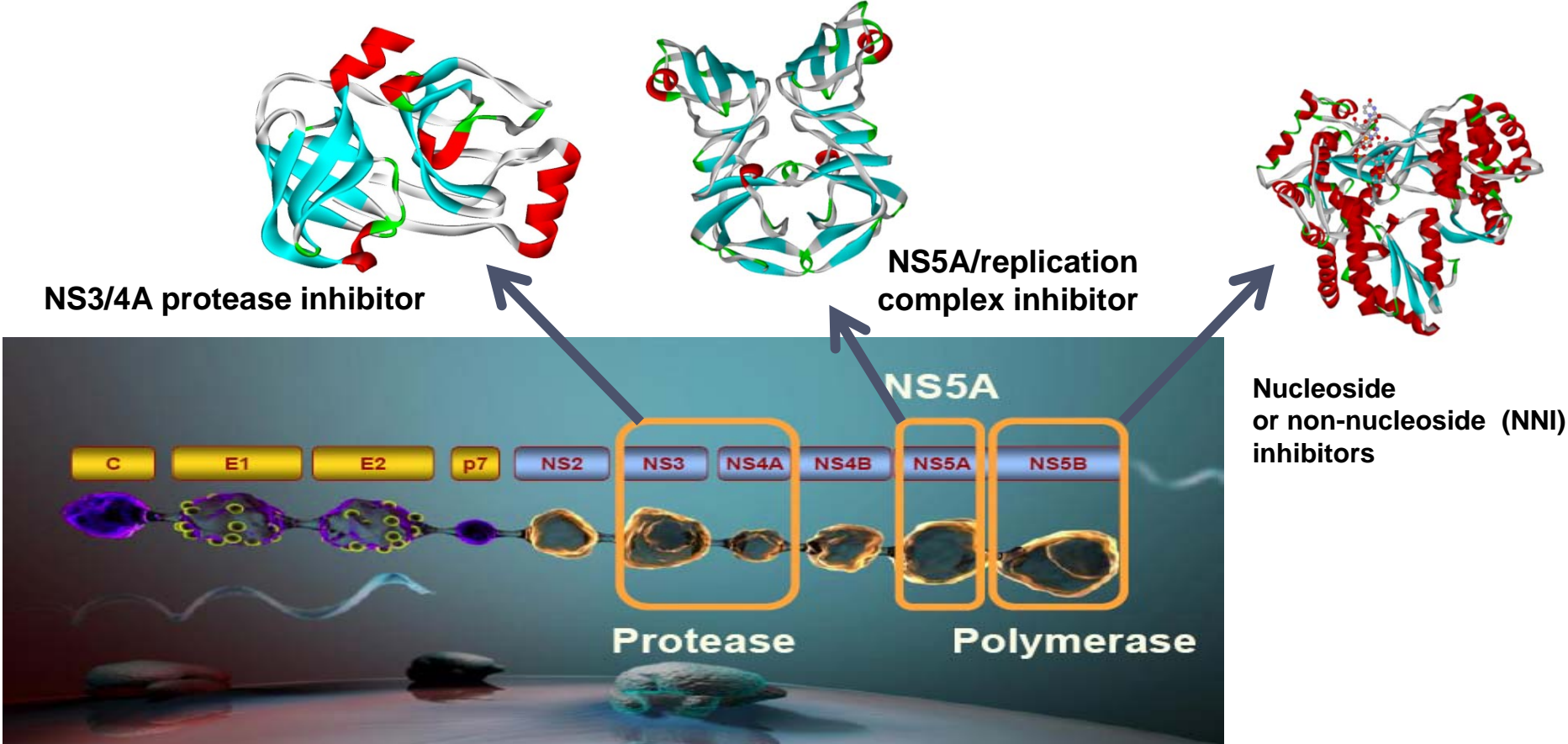
## Excellent efficacy, safety and tolerability

- **~80 % overall cure rates** (up to 91% of patients could stop all treatment at 24 weeks)
- **Overall incidence of adverse events similar to placebo**





# Three major targets in hepatitis C virus

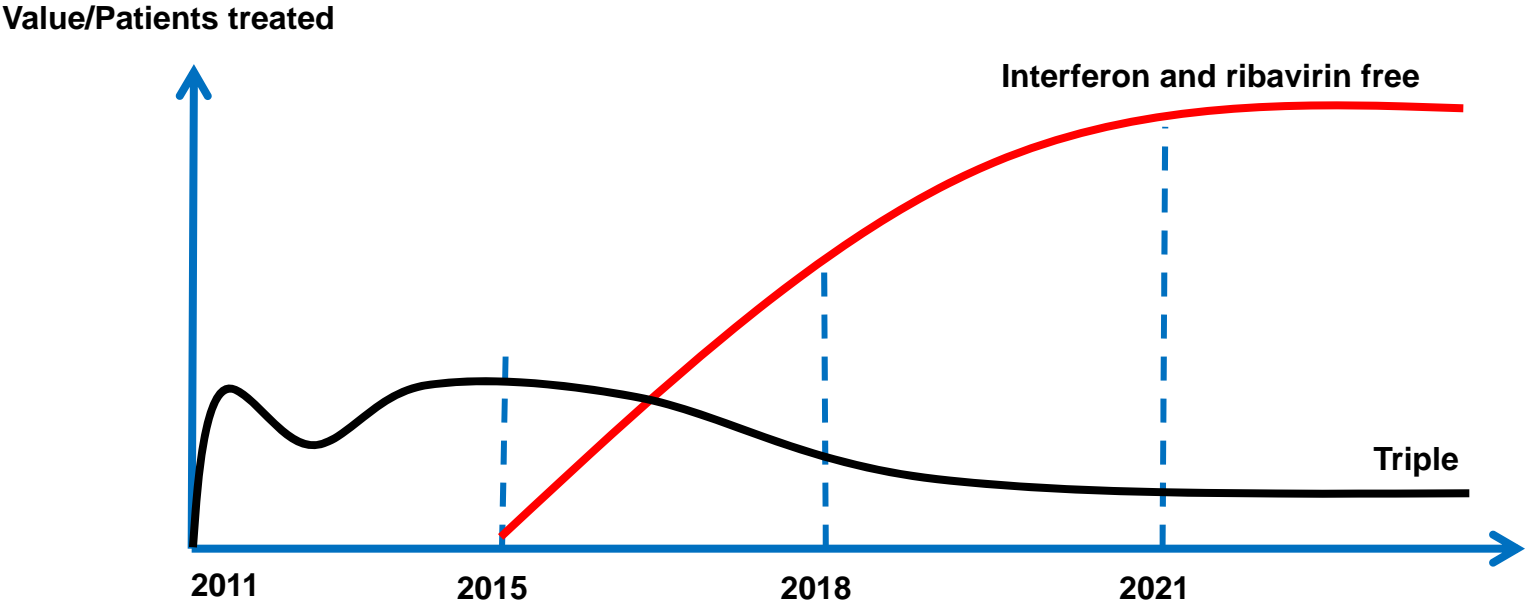


# Long term objective - eradication of hepatitis C










The evolution in treating hepatitis C will expand the market value, number of patients treated and regions over the next 10-15 years

Market value, peak sales >20 BUSD



# Simeprevir in interferon-free combinations

Ribavirin

<b>Simeprevir</b>	<b>+</b>	<b>Sofosbuvir</b> (nucleotide)	+/-		N= 80 Cohort 1: nulls, N= 87 Cohort 2: nulls + naives with METAVIR score <b>F3 and 4 only</b>
			+/-		
<b>Simeprevir</b>	<b>+</b>	<b>Daclatasvir</b> (NS5A inhibitor)	+/-		N= 180 Naives and nulls <b>Including F3 and 4 patients</b>
			+/-		
<b>Simeprevir</b>	<b>+</b>	<b>TMC647055/r</b> (NNI; non-nucleoside)	+/-		Naives/relapsers and nulls
<b>Simeprevir</b>	<b>+</b>	<b>VX-135</b> (nucleotide)	+/-		Phase II to start H2, 2013 - on track
<b>Simeprevir</b>	<b>+</b>	<b>IDX719</b> (NS5A inhibitor)	+/-		HELIX-1, Phase II started Q2 HELIX-2, to start during Q3
		<b>+/- TMC647055/r</b>			

**Simeprevir is strongly positioned to become a principal component of future IFN-free therapies**



# **COSMOS Study**

(interim analysis)

**Once-daily regimen of simeprevir plus  
sofosbuvir with or without ribavirin in hard  
to cure HCV patients \***

\*The **COSMOS** study: **CO**mbination of **SiM**eprevir and **sO**fosbuvir in HCV infected patients**S**

# COSMOS study – Summary of Interim Results: Efficacy

Efficacy results with simeprevir (SMV) and sofosbuvir (SOF) once daily for 12 weeks with or without ribavirin (RBV).

	Cohort 1		Cohort 2	
	<u>Prior null responder</u> HCV patients (METAVIR score F0-F2)		<u>Prior null responder and treatment naïve</u> HCV patients (METAVIR scores F3 or F4)	
	SMV / SOF+ RBV (n=27)	SMV / SOF (n=14)	SMV / SOF + RBV (n=27)	SMV / SOF (n=14)
SVR4	26/27 (96%)	13/14 (93%)	26/27 (96%)	14/14 (100%)
SVR8	26/27 (96%)	13/14 (93%)	-	-

- Interim results indicate high efficacy in hardest to cure HCV patients
- Once-daily simeprevir and sofosbuvir was generally safe and well tolerated

Sustained Virologic Response 4 or 8 weeks (SVR4 or SVR8) after end of treatment.

# Value proposition – the road towards profitability

Area	Project	Partner	Preclinical phase				Clinical phase				Status
			Phase 1	Phase 2	Phase 3	Phase 4	Phase 1	Phase 2	Phase 3	Phase 4	
Antivirals	Levatinervir (Dolutegravir, Zidovudine, Zalcitabine)	Janssen	Phase 1	Phase 2	Phase 3	Phase 4					Approved
	Isosiprevir (Simeprevir)	Janssen	Phase 1	Phase 2	Phase 3	Phase 4					Approved
	Isosiprevir (Simeprevir)	Janssen	Phase 1	Phase 2	Phase 3	Phase 4					Approved
	Isosiprevir (Simeprevir)	Janssen	Phase 1	Phase 2	Phase 3	Phase 4					Approved
HIV	Isosiprevir (Simeprevir)	Janssen	Phase 1	Phase 2	Phase 3	Phase 4					Approved
	Isosiprevir (Simeprevir)	Janssen	Phase 1	Phase 2	Phase 3	Phase 4					Approved
Other indications											
Respiratory	Campan 1 inhibitor										
Neurology	Campan 2 inhibitor										

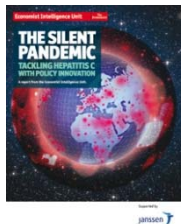


## Innovative portfolio will evolve over time

- World class expertise in polymerase and protease drug targets
- Commitment towards targets in infectious diseases
- New therapeutic areas based on core competence
- Partner of choice for pharmaceuticals and development programs

## Long term commitment in the HCV area

- Simeprevir, partnered with Janssen Pharmaceuticals
  - Regulatory files submitted in EU, US and Japan
  - Ongoing interferon-free combination trials will guide treatment opportunities
- In-house unpartnered HCV nucleotide-based polymerase inhibitor program can offer new combination treatment opportunities



# Value proposition – the road towards profitability



## Commercial presence in the Nordic region creates revenue

- 15 solid Rx pharmaceuticals with annual sales of ~170 MSEK
- Commercial platform for the Nordic launch of simeprevir in 2014
- Expansion of product portfolio



## External perspective

- Top ranked as a listed company
- Profitable and fast growing Nordic based pharmaceutical company

# Key events in the coming 12 month

Part	Project	Partner	Preclinical phase			Clinical phase			Market
			No. of studies	Phase 1	Phase 2	Phase 3	Phase 4		
<b>Antivirals</b>									
Label transfer	laniniraparivir (laniniraparivir, Simeprevir)	Daichi Sankyo (DS)							
Hepatitis C	simeprevir (TMC647055)	Janssen							
Hepatitis C	NS5B protease inhibitor	Pharmaceuticals							
Hepatitis B	NS5B nucleoside inhibitor (MIV-711)	Genzyme							
Hepatitis C	NS5B nucleoside based protease inhibitor	Janssen							
Hepatitis C	NS5B nucleoside based protease inhibitor	Pharmaceuticals							
Hepatitis C	NS5B nucleoside based protease inhibitor	Janssen							
MIV	Protease inhibitor	Janssen							
MIV	Protease inhibitor	Pharmaceuticals							
<b>Other indications</b>									
Bone related disorders	Cathepsin K inhibitor								
Neuropathic pain	Cathepsin S inhibitor								

H2-13 Results from phase I-study with MIV-711, our cathepsin K inhibitor (bone related disorders)

H2-13 Start of the phase II study - HELIX-2 (simeprevir + TMC647055 and samatasvir - IDENIX)

H2-13 Start of Phase II with simeprevir and VX-135 (Vertex)

H2-13 Potential CD selection in Cathepsin S (neuropathic pain) program

H2-13 Goal to start phase I trials with Medivir/Janssen nucleotide NS5B-inhibitor

H2-13 Presentations at AASLD

H2-13 SVR data from phase II Cosmos study with simeprevir and sofosbuvir

H2-13 Anticipated approval in Japan for simeprevir

H2-13 Anticipated approval of simeprevir in the US

H2-13 Data from the phase II combination study with simeprevir and daclatasvir (BMS)

H1-14 Anticipated approval of simeprevir (triple) in EU

H1-14 Presentations at EASL

H1-14 Potential CD selection in our internal Nucleotide NS5B inhibitor program



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

**Ticker: MVIR**  
**Exchange: OMX / NASDAQ**

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