



Medivir

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

EASL Medivir presentation 27 April

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Value proposition – a platform for growth and profitability

Innovative portfolio that will evolve over time

- World class expertise in polymerase and protease drug targets
- R&D focus on infectious diseases

Strong position in HCV – goal is take part in eradicating hepatitis C

- Simeprevir, partnered with Janssen Pharmaceuticals
 - Regulatory files have been submitted in EU, US and Japan
 - Many interferon-free combination treatments opportunities
- In-house HCV programs will offer new combination opportunities

Commercial presence in the Nordic region creates stability

- Solid brand names with annual sales of ~85 MUSD
- Commercial platform for the launch of simeprevir in the Nordics in 2014
- Pharmaceutical portfolio will be broadened

BioPhausia
– a Medivir sales company

2013 - Setting the framework for becoming *The Emerging European Pharma Company*

Structure

- Broader, risk balanced, R&D pipeline
- Continued commitment towards targets in infectious diseases
- Addressing new therapeutic areas based on core competence

Therapeutic area	Product/Target	Partner	Preclinical phase				Clinical phase				
			Research	Phase I	Phase II	Phase III	Phase I	Phase II	Phase III	Market	
ANTIVIRALS	Leads/Target	Merck/Novartis, Novartis, Daxx	█	█	█	█	█	█	█	█	█
	Regeneron C	Novartis (HIV-1), NCI, Johnson & Johnson	█	█	█	█	█	█	█	█	█
	MSD	MSD	█	█	█	█	█	█	█	█	█
	MSD	MSD	█	█	█	█	█	█	█	█	█
Regeneron B	MSD	█	█	█	█	█	█	█	█	█	
Daxx/Novartis	Novartis	█	█	█	█	█	█	█	█	█	
MSD	MSD	█	█	█	█	█	█	█	█	█	
OTHER INDICATIONS											
Beta-actin	Cellcept, K. inhibitor		█	█	█	█	█	█	█	█	█
Alendronate	Cellcept, K. inhibitor		█	█	█	█	█	█	█	█	█
Neuroprotection	Cellcept, K. inhibitor		█	█	█	█	█	█	█	█	█

- Partner of choice for both pharmaceuticals and development programs
- Expansion of product portfolio, including simeprevir and other in-house developed pharmaceuticals



External perspective

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

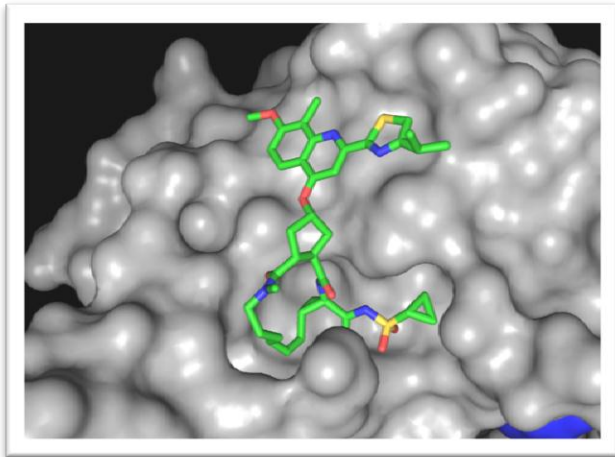




Simeprevir – An update

Charlotte Edenius

Simeprevir



One-pill, once-daily, investigational, oral HCV NS3/4A protease inhibitor

- Antiviral activity against HCV GT 1, 2, 4, 5, and 6
- Safe and well tolerated in clinical trials, with high SVR rates
- Three large pivotal phase III studies of SMV 150 mg QD plus PegIFN/RBV completed (QUEST-1 and QUEST-2 in treatment-naïve and PROMISE in prior relapsers)
- Simeprevir is currently being studied in a number of IFN-free regimens, including the COSMOS study

Simeprevir - clinical development programs in HCV G1 & 4 infected patients

Pivotal phase III studies:

- **QUEST 1** treatment-naïve
- **QUEST 2** treatment-naïve
- **PROMISE** prior relapsers
- **Japan** naïve & experienced (four studies)

Regulatory files submitted in US (March-13)
and in EU (April-13)

Regulatory file submitted February 2013

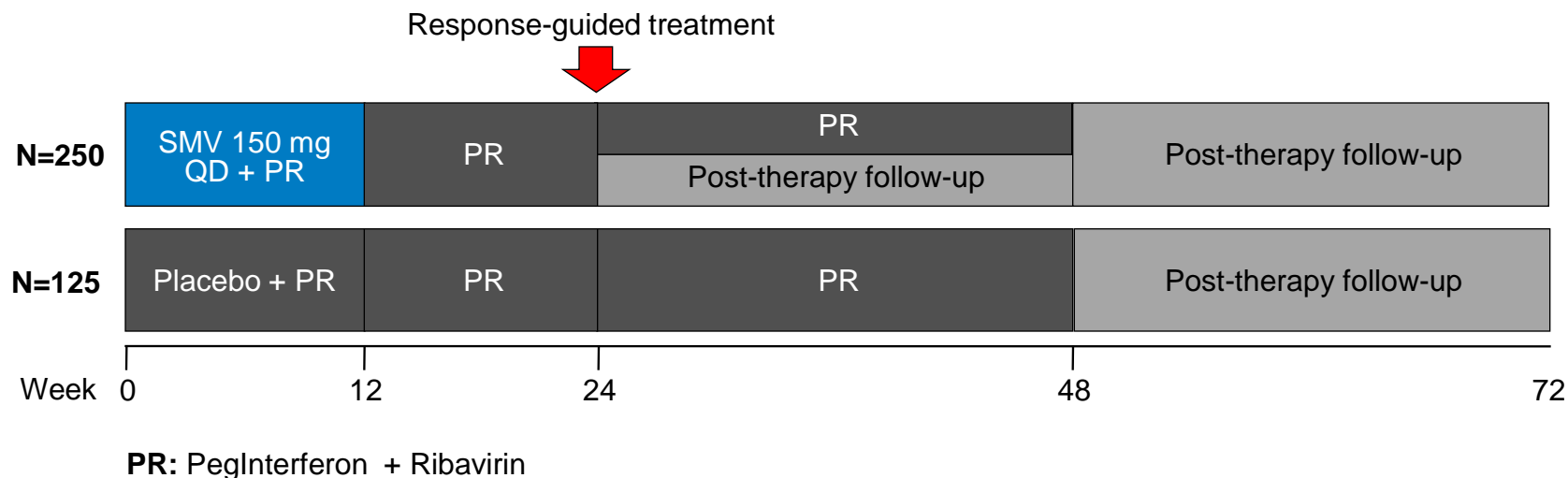
Other ongoing phase III studies:

- **China:** Efficacy, PK, safety and tolerability in naïve patients
- **ATTAIN:** Simeprevir vs telaprevir in prior null or partial responders
- **RESTORE:** HCV genotype 4 infected naïve or treatment experienced patients
- **C212:** HIV-HCV co-infected treatment naïve and treatment experienced patients

Regulatory filings for simeprevir triple combination submitted in US, EU & Japan

Simeprevir - Phase III Study designs in HCV GT1 infected patients (QUEST-1, QUEST-2 and PROMISE)

- Randomized, double-blind, placebo controlled design
- Patients were stratified by HCV subtype and IL28B genotype



Studies:

- QUEST-1: n=394, naive (METAVIR score F3-F4: 30%)
- QUEST-2: n=391, naive, (METAVIR score F3-F4: 22%)
- PROMISE: n=393, prior relapsers, (METAVIR score F3-F4: 31%)

Top line data
Dec-12

Reported at EASL 2013

Summary: SVR12 data from QUEST-1 and QUEST-2

Patients with SVR12 %	QUEST-1		QUEST-2	
	SMV + PR	Placebo + PR	SMV + PR	Placebo + PR
All patients	80	50	81	50
SVR12 with 24 wks treatment (% pat meeting RGT criteria)	91 (85)	N/A	86 (91)	N/A
CC / CT / TT	94 / 76 / 65	78 / 42 / 24	96 / 80 / 58	81 / 41 / 19
GT1a & other / GT1b	71 / 90	49 / 52	80 / 82	46 / 53
F0-F2	83	60	85	51
F3-F4	70	28	66	47

Robust data from two large placebo-controlled studies including a large proportion of patients with advanced liver fibrosis (F3-F4)

Summary: adverse events QUEST-1 and QUEST-2 across all treatment phases

Patients, %	QUEST-1		QUEST-2	
	SMV/PR (N=257)	PBO/PR (N=134)	SMV/PR (N=257)	PBO/PR (N=134)
Most common AEs ($\geq 25\%$ in SMV arm)				
Fatigue	42	41	37	42
Pruritus	26	16	25	25
Headache	33	39	39	37
Pyrexia			31	40
Influenza-like illness			26	26
Other AEs of interest				
Rash (any type)	34	32	27	20
Anemia	20	28	21	28
Photosensitivity*	3	1	4	1

* Over the first 12 weeks treatment

Overall incidence of adverse events was similar to placebo control

Simeprevir - Phase III triple therapy

Summary

Robust efficacy with high cure rates (SVR12):

- Naive and relapser patients in three large global studies: 79-81%¹
- Confirmed in Japan program, where high cure rates were demonstrated²
 - ✓ Broad filing on treatment naive and non-responders

High cure rates with 24 weeks treatment duration

- 85-93% stopped all treatment at 24 weeks (QUEST-1 & -2 and PROMISE)
- High SVR12 rates 86-91% (QUEST-1 and -2, PROMISE data to be presented)

Excellent safety and tolerability

- Overall incidence of adverse events similar to placebo, including rash and anemia
- Confirmed in Japanese studies

Regulatory filings for simeprevir triple combination submitted in US, EU & Japan

Simeprevir - clinical development programs in HCV G1 & 4 infected patients

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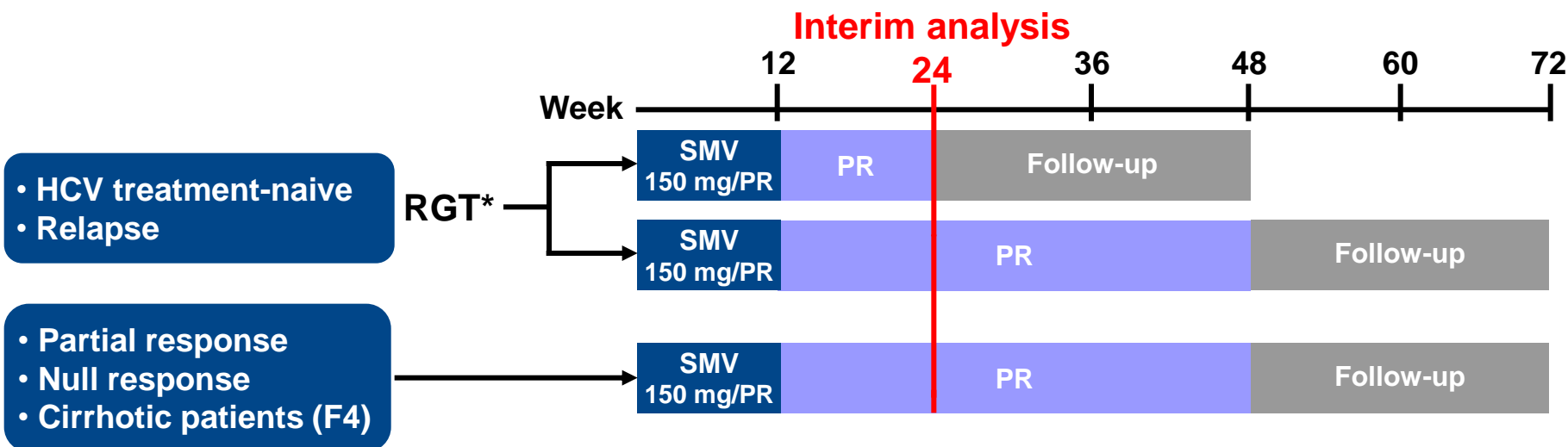
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C212 HCV-HIV Co-infected

Study design



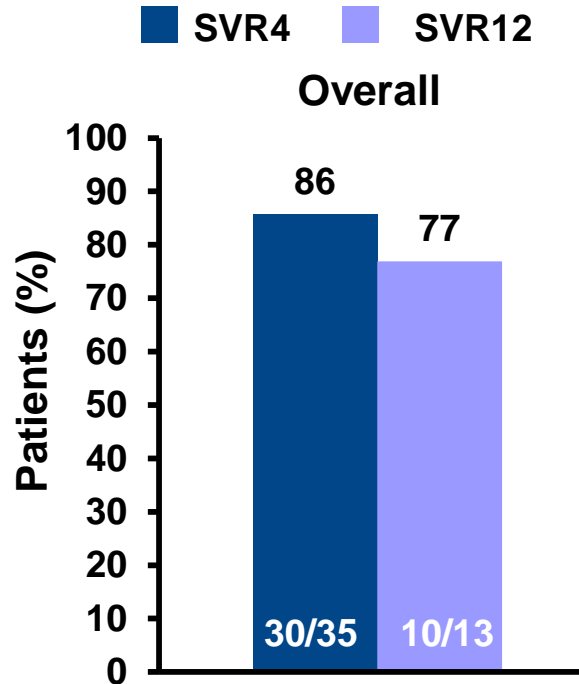
Interim analysis:

➤ All patients included in the analysis had completed 24 weeks of treatment or had discontinued prior to that point

➤ No. of patients: Week 24: N=100
Week 28: N=71
Week 36: N=27

C212: HCV-HIV Co-infected

Preliminary SVR4 and SVR12 rates in patients treated with SMV/PR



- 82% GT1a,
- 21% (METAVIR F3/4)
- 93 out of 106 patients on ARV therapy

- 88% met RGT criteria and stopped all treatment at W24

- Simeprevir was safe and well tolerated
- No HIV breakthroughs

In the US 25 % of HIV patients are coinfectd with HCV



Simeprevir

- All oral interferon free combination update

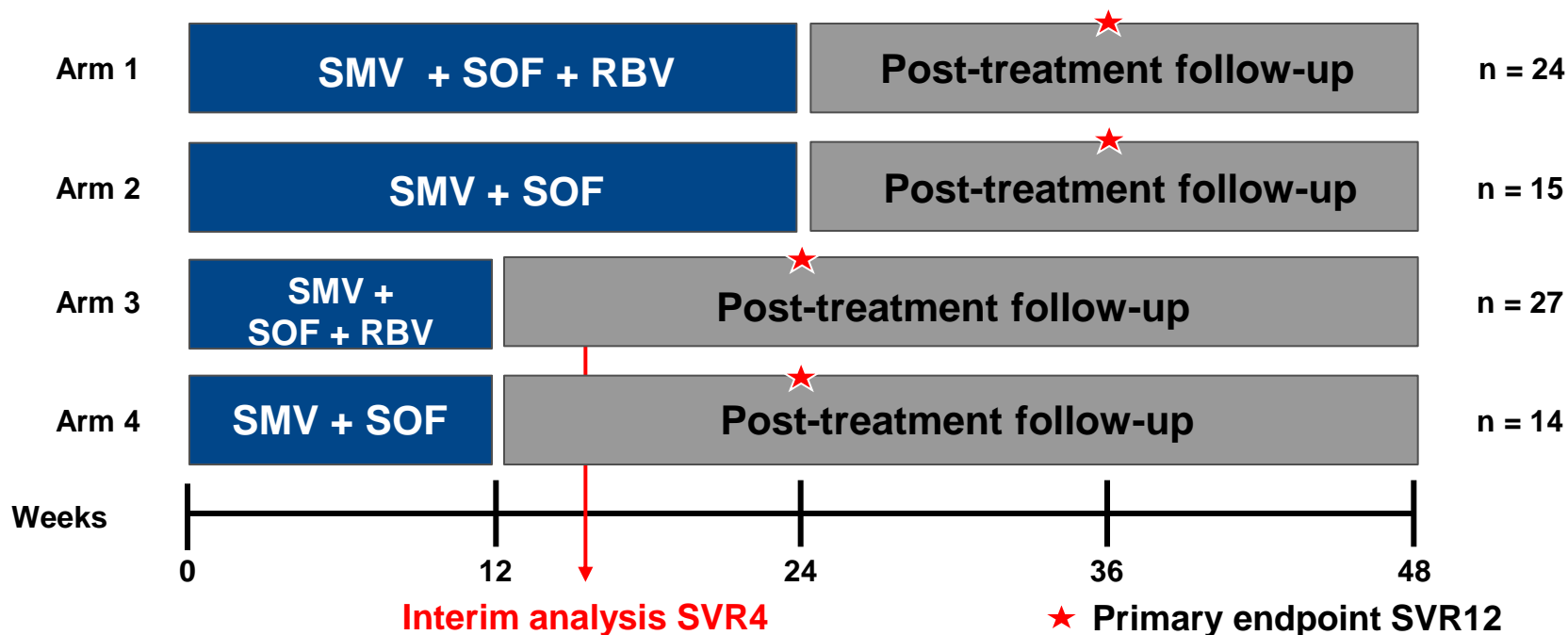


COSMOS Study

(interim analysis)

Once-daily regimen of simeprevir plus sofosbuvir with or without ribavirin in HCV genotype 1 null responders*

COSMOS study - Design



- Cohort 1: n=80, nulls, F0-F2
- Cohort 2: n=87, naives and nulls, F3-F4
- SMV 150 mg QD + SOF 400 mg QD with/without RBV
- Interim analysis of Cohort 1 conducted when all patients in 12 week treatment arms reached SVR4 time point or discontinued early

COSMOS study - Baseline patient demographics and HCV disease characteristics

Patients		Total (n=80)
Patient demographics		
Male		61%
Race	Caucasian	71%
	African American	29%
Ethnicity	Hispanic/Latino	25%
Age, years, median		56.0
BMI, kg/m ² , median		27.5
<i>IL28B</i>	nonCC	94%
Baseline characteristics		
HCV subtype	1a	78%
HCV RNA, median, log ₁₀ IU/mL		6.8
METAVIR score	F0-F1	41%
	F2	59%

COSMOS study – Efficacy results (interim analysis)

Patients	24 weeks		12 weeks	
	SMV + SOF +RBV (n=24)	SMV + SOF (n=15)	SMV + SOF+ RBV (n=27)	SMV + SOF (n=14)
RVR ¹ , n/N (%)	18/22 (81.8)	10/15 (66.7)	23/27 (85.2)	8/14 (57.1)
Undetectable end of treatment, n/N (%)	10/12 (83.3)	8/9 (88.9)	27/27 (100)	14/14 (100)
Relapse, n	0	0	1	1
SVR ₄ , n/N (%)	4/6 (66.7)	5/5 (100)	26/27 (96.3)	13/14 (92.9)
SVR₈, n/N (%)	4/6 (66.7)	5/5 (100)	26/27 (96.3)	13/14 (92.9)

Of the patients in the **12 week arms** who achieved SVR₈
 – **24/24** who reached post-treatment Week 12 had achieved **SVR₁₂**

COSMOS study - Summary & Conclusions

- SMV + SOF with or without RBV for 12 weeks yielded high SVR rates in prior null responders with mild to moderate fibrosis (METAVIR F0-F2)
 - ✓ **SVR8 rate of 96% with RBV and 93% without RBV**
- SMV + SOF was safe and well tolerated
 - ✓ Anemia was seen only in RBV arms
 - ✓ Bilirubin increases only occurred in RBV containing arms

Enrollment of Cohort 2 is complete and include prior null responders and treatment-naïve patients all with advanced fibrosis (METAVIR F3-F4)

Simeprevir in interferon-free combinations

Ribavirin

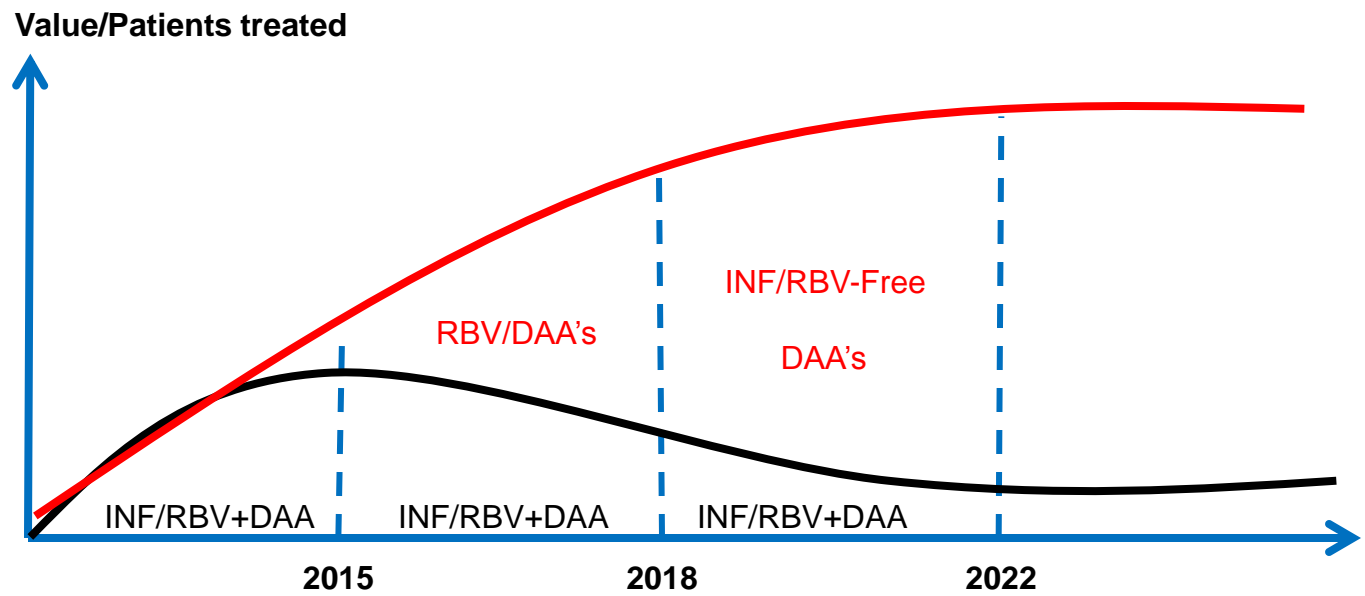
Simeprevir	+	Sofosbuvir (nucleotide)	+/-	12w	N=80+87 ✓ Cohort a: nulls, F0-F2 Cohort b: nulls + naives; F3/4 (cirrhotics)
			+/-	24w	
Simeprevir	+	Daclatasvir (NS5A inhibitor)	+/-	12w	N=180 Naives and nulls Incl. F3/4 up to 35 %
			+/-	24w	
Simeprevir	+	TMC647055/r (NNI; non-nucleoside)	+/-	12w	Naives/relapser and nulls Non-cirrhotics
Simeprevir	+	VX-135 (nucleotide)	+/-	12w	Phase II to start H1 2013
Simeprevir	+	IDX719 (NS5A inhibitor) +/- TMC647055/r	+/-	12w	DDI study started

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

Long term goal – 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



News flow - highlights

Therapeutic area	Product/Project	Partner	Preclinical phase		Clinical phase			Market	
			Research	Development	Phase I	Phase IIa	Phase IIb		Phase III
ANTIVIRALS									
Labial herpes	Xerclear® (Zovirax, Zovirax Duo)	GlaxoSmithKline (GSK)	[Progress bar]						
	Simeprevir (TMC435), NS3 protease inhibitor	Janssen Pharmaceuticals	[Progress bar]						
Hepatitis C	NS5B nucleotide polymerase inhibitor	Janssen Pharmaceuticals	[Progress bar]						
	NS5B nucleotide polymerase inhibitor	Janssen Pharmaceuticals	[Progress bar]						
Hepatitis B	NS5A replication complex inhibitor	Janssen Pharmaceuticals	[Progress bar]						
	Lagociclovir valactate (MIV-210)	Daewoong	[Progress bar]						
Dengue fever	NS3 protease inhibitor	Janssen R&D Ireland	[Progress bar]						
HIV	Protease inhibitor	Janssen Pharmaceuticals	[Progress bar]						
OTHER INDICATIONS									
Bone related disorders	Cathepsin K inhibitor		[Progress bar]						
Neuropathic pain	Cathepsin S inhibitor		[Progress bar]						

- ✓ Q4-12 Start of Cohort 2 with simeprevir and GS7977 phase II study
- ✓ Q4-12 Top line results from phase III trials with simeprevir (Quest 1+2 and Promise)
- ✓ Q1-13 Filing for regulatory approval in Japan
- ✓ H1-13 EoT and partial SVR data from Cohort 1 with simeprevir and sofosbuvir phase II study
- ✓ H1-13 Filing of simeprevir in Japan, the US and EU
- H1-13 Potential CD selection in Cathepsin S (neuropathic pain) program
- H1-13 Results from phase I-study with MIV-711, our cathepsin K inhibitor (bone related disorders)
- H1-13 Start of Phase II with simeprevir and VX-135
- H1-13 Step two in GSK launch strategy for Xerclear® (ZoviDuo), launch in major European OTC markets
- H2-13 Potential CD selection in our internal Nucleotide NS5B inhibitor program
- H2-13 Potential CD selection in our internal NS5A inhibitor program
- H2-13 Goal to start phase I trials with Medivir/Janssen nucleotide NS5B-inhibitor
- H2-13 Data from the phase II combination study with simeprevir and daclatasvir
- H2-13 SVR data from Cohort 2 with simeprevir and sofosbuvir phase II study

www.medivir.com

Ticker: MVIR
Exchange: OMX / NASDAQ

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