



# Medivir

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

**Carnegie Health Care Seminar March 2013**

**Maris Hartmanis, CEO**

# 2012 – Summary

## R&D operations

- Progress in R&D pipeline, both internally driven and partnered projects
- Simeprevir phase III data showed strong and consistent results, followed by filing in Japan
- Broadening of research platform and know-how through new collaborations and an acquisition

## Pharmaceuticals

- Consistent product portfolio performance, earnings in line with expectations at the acquisition in 2011, with a EBITDA contribution of ~100 MSEK
- GSK started OTC launch in Europe and obtained OTC approval in Russia with the Medivir developed cold sore pharmaceutical branded as Zoviduo/Zovirax Duo
- Preparations and awareness building around simeprevir in the Nordics made strong progress

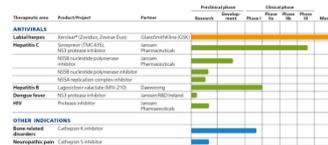
## Finance

- Solid financial position at year end with ~300 MSEK in cash
- Stable cost base with a net burn rate of ~200 MSEK

# 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



- 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

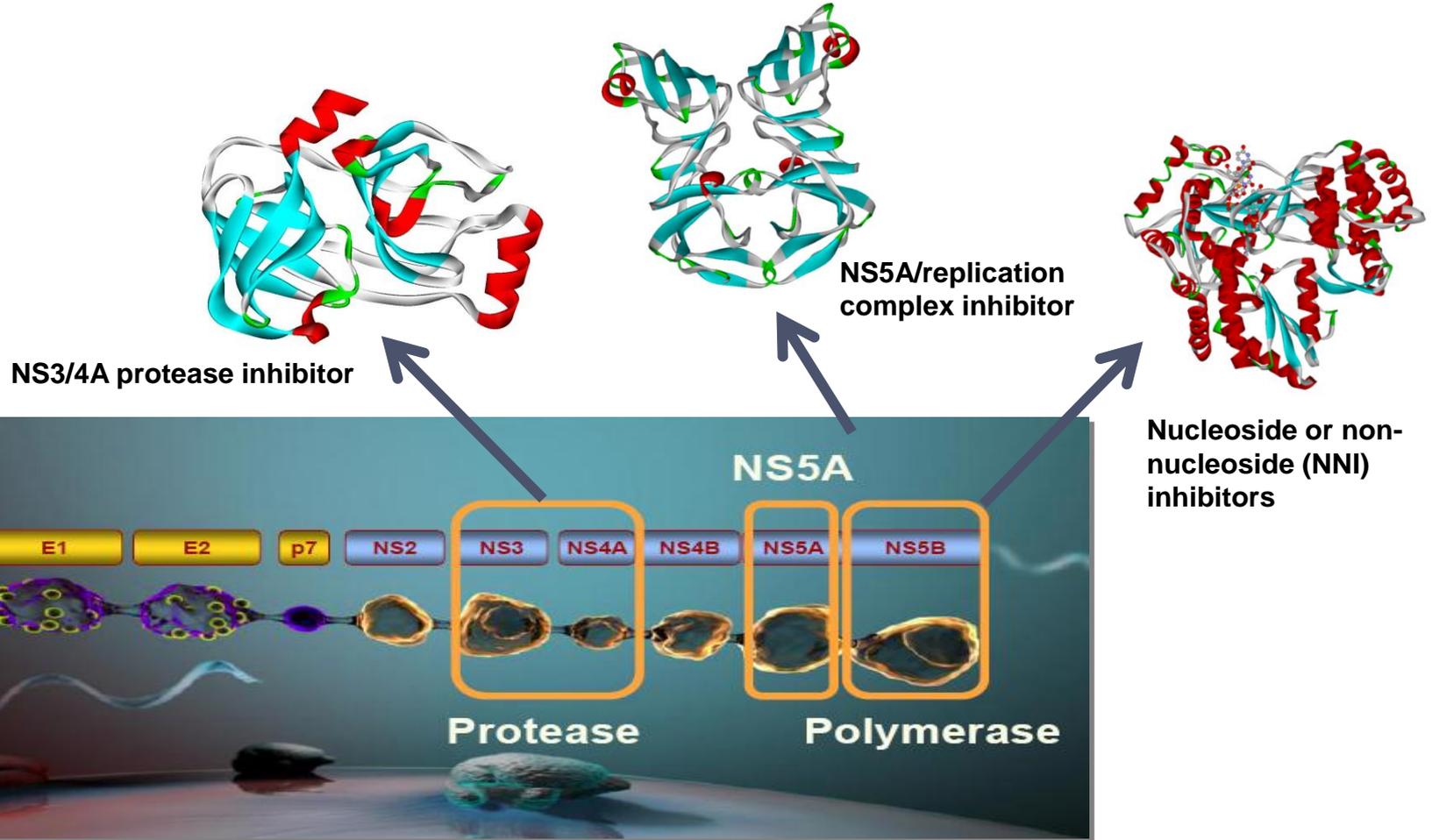


# The R&D portfolio will evolve over time

- New infectious disease research programs underway
- New therapeutic areas will be evaluated

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

# Our commitment in hepatitis C



**Simeprevir – An efficacious, safe and tolerable protease inhibitor\***

# Simeprevir - clinical development programs in HCV G1 & G4 infected patients

## Pivotal phase III studies:

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

← Top-line data available

← Regulatory file submitted Feb. 22, 2013

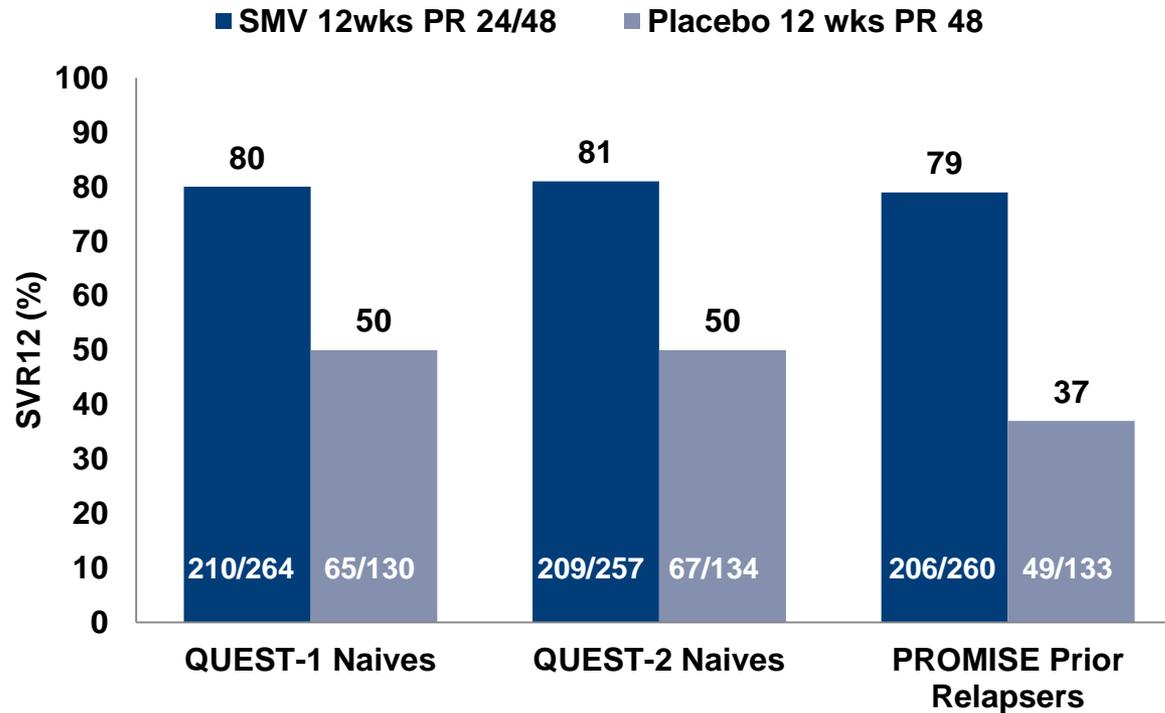
## Other ongoing phase III studies:

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

Regulatory filings for simeprevir triple combination H1, 2013 in US, EU & Japan

# Simeprevir - Phase III triple therapy

## Efficacy – SVR12 (cure rate)



Statistically significant difference vs placebo control in all studies

Robust efficacy in all three studies (79-81% SVR12) confirming phase II studies

# Simeprevir - Phase III triple therapy (global and Japan)

## Summary

### **Robust efficacy with high cure rates (SVR12):**

- Naive and relapser patients in three global studies: 79-81%<sup>1</sup>
- Confirmed in Japan program, where high cure rates were demonstrated<sup>2</sup>

### **Shorter treatment duration**

- 85-93% could stop all treatment at week 24 (naïve and relapser patients; global trials)

### **Excellent safety and tolerability**

- Overall incidence of adverse events, including rash and anemia, similar to placebo
- Confirmed in Japan program, where favourable safety profile was demonstrated

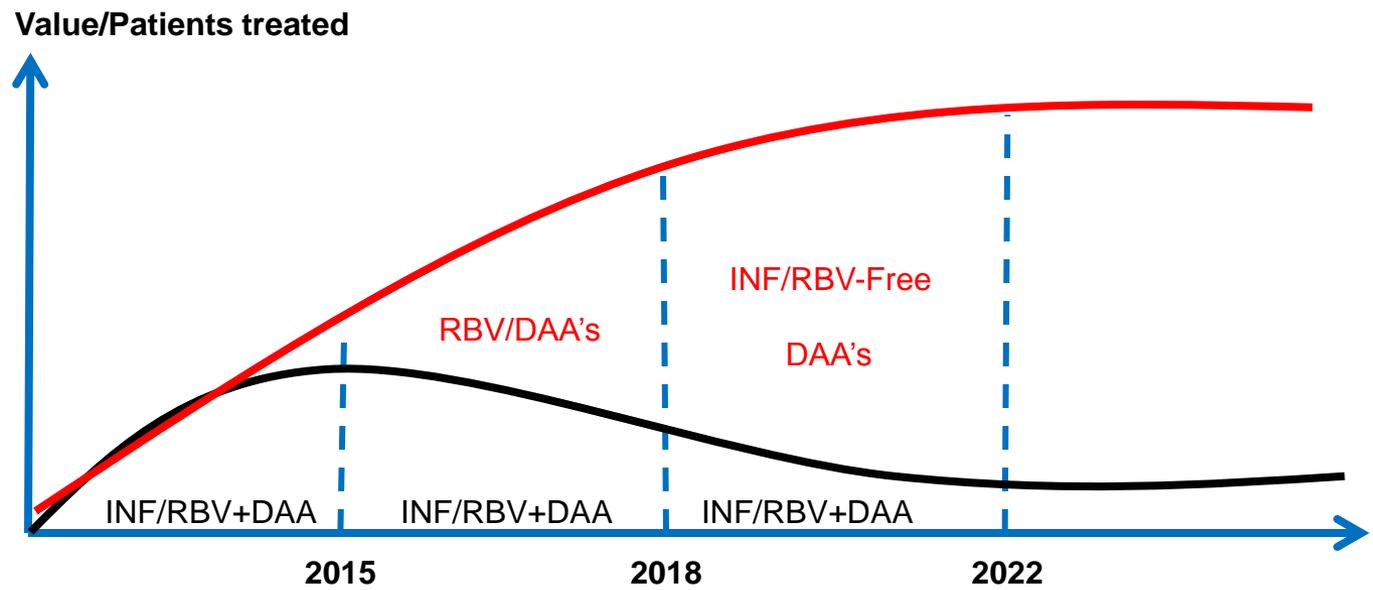
**Phase III data support simeprevir as a new treatment for G1 HCV, with advantages versus marketed 1<sup>st</sup> generation protease inhibitors**

**Regulatory filings for simeprevir triple combination H1, 2013 in US, EU & Japan**

# Long term goal – eradication of hepatitis C

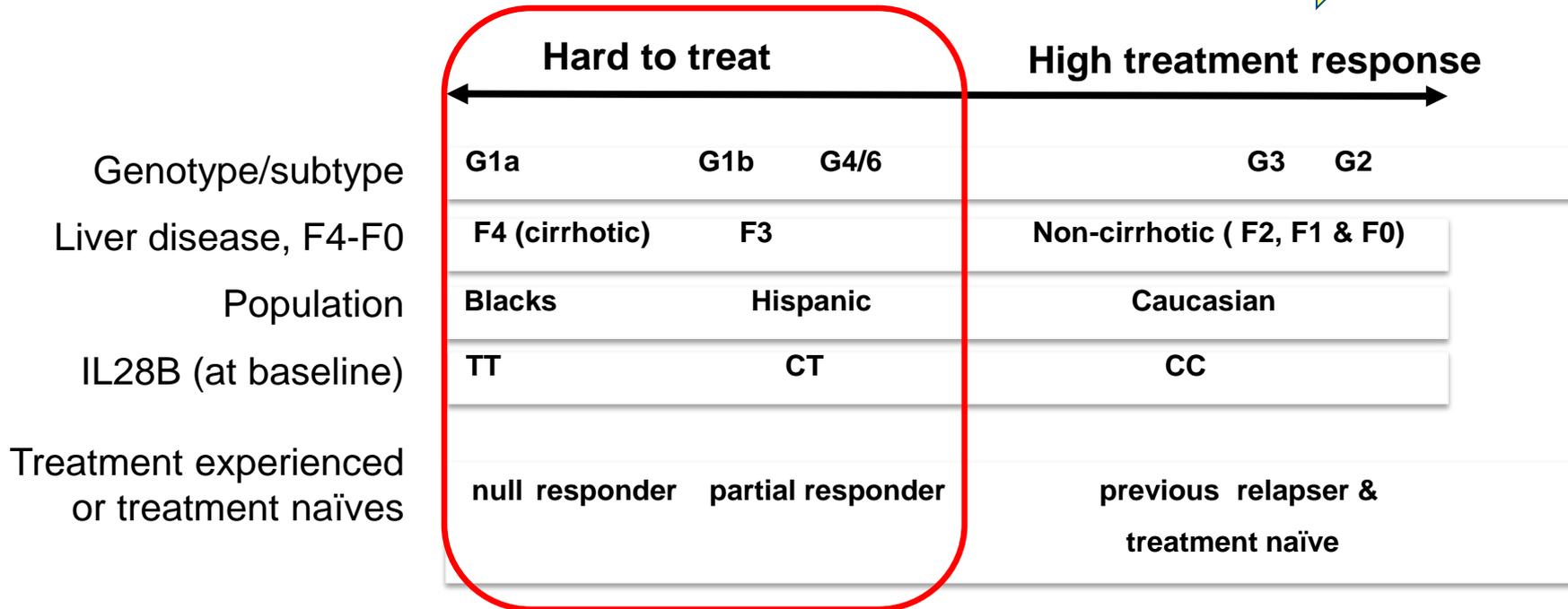


The evolution in treating hepatitis C will expand the market value, number of patients treated and regions over the next 10-15 years



# Patient response to treatment – a complex picture

Proof of concept starts in difficult to treat patients 

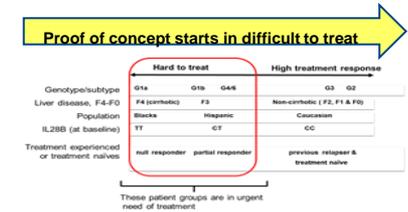


These patient groups are in urgent need of treatment

**Simeprevir has demonstrated efficacy in difficult to treat G1 patients with severe liver disease**

# Interferon-free combinations in HCV null responders

- Prior null responders to pegIFN/RBV have limited treatment options
- PegIFN/RBV-containing treatments are difficult to tolerate and contraindicated in many patients
- All patients without cirrhosis



Danoprevir/r + Mericitabine + RBV	Roche	<b>55% SVR12 (GT 1b)</b>	
Daclatasvir + asunaprevir	BMS	<b>64- 91% SVR12 (GT 1b)</b>	24 week duration
ABT-450/r + ABT-267 + RBV	Abbott	<b>89% SVR12</b>	
ABT-450/r + ABT-267 + ABT-333 + RBV		<b>93% SVR12</b>	
ABT-450/r + ABT-333 + RBV		<b>47% SVR12</b>	
Sofosbuvir + RBV	Gilead	<b>10% SVR12</b>	
Sofosbuvir + ledipasvir + RBV		<b>100% SVR12 (9/9 patients)</b>	
Simeprevir + Sofosbuvir +RBV	Medivir/J&J	<b>97% SVR8 (26/27 patients)</b>	
Simeprevir + Sofosbuvir		<b>93% SVR8 (13/14 patients)</b>	

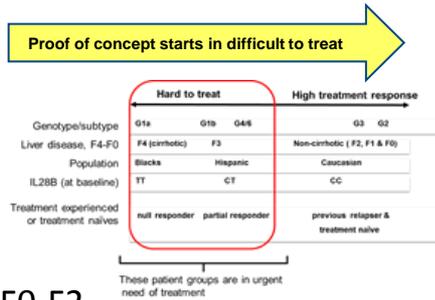
# COSMOS phase III-study: Efficacy results

Patients	24 weeks		12 weeks	
	SMV + SOF + RBV	SMV + SOF	SMV + SOF + RBV	SMV + SOF
RVR <sup>1</sup> , 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 (100.0)	14/14 (100.0)
Relapse, n	0	0	1	1
SVR <sub>4</sub> , n/N (%)	4/6 (66.7)	5/5 (100.0)	26/27 (96.3)	13/14 (92.9)
SVR <sub>8</sub> , n/N (%)	4/6 (66.7)	5/5 (100.0)	26/27 (96.3)	13/14 (92.9)

Of the patients in the 12 week arms who achieved SVR<sub>8</sub>

- 24/24 who reached post-treatment Week 12 had undetectable HCV RNA (SVR<sub>12</sub>)
- 8/8 who reached post-treatment Week 24 had undetectable HCV RNA (SVR<sub>24</sub>)

# Simeprevir in interferon-free combinations



**Simeprevir + Sofosbuvir** (nucleotide) +/- **12w**  
 +/- **24w**

N=80+87  
 ✓ Cohort a: nulls, F0-F2  
 Cohort b: nulls + naives; **F3/4 (cirrhotics)**

**Simeprevir + Daclatasvir** (NS5A inhibitor) +/- **12w**  
 +/- **24w**

N=180  
 Naives and nulls  
**Incl. F3/4 up to 35 %**

**Simeprevir + TMC647055/r** (NNI; non-nucleoside) +/- **12w**

Naives/relapser and nulls  
 Non-cirrhotics

**Simeprevir + VX-135** (nucleotide) +/- **12w**

DDI study to start Q1, 2013

**Simeprevir + IDX719** (NS5A inhibitor) +/- **12w**  
 +/- TMC647055/r

DDI study started (simeprevir + IDX719)

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

# 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 filing began already in Q1, 2013 as a triple combination treatment with PegIFN and ribavirin
  - 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

# News flow - highlights



- ✓ 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 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 study 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](http://www.medivir.com)

**Ticker: MVIR**

**Exchange: OMX / NASDAQ**

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