# A Focused Strategy for Sustainable Value Creation

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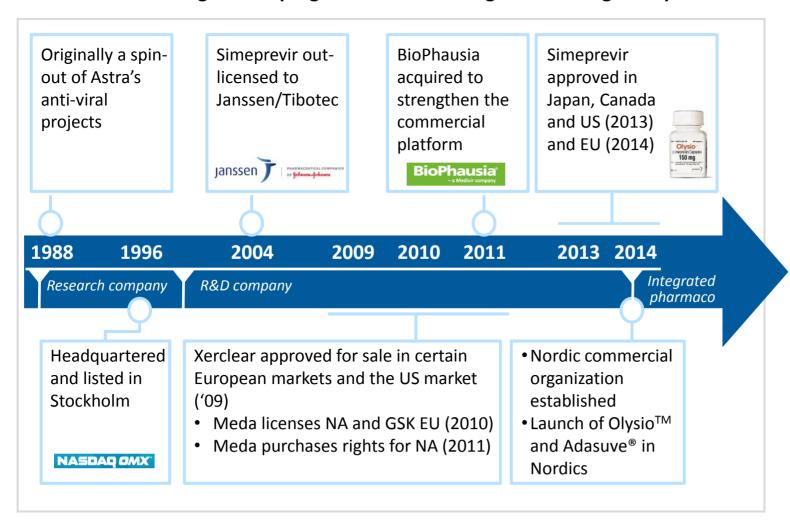


A Nordic research-based pharmaceutical company focused on infectious diseases and oncology

## Medivir is a Nordic research-based pharma company focused on infectious diseases and oncology (1/2)



### Medivir has made significant progress in transforming into an integrated pharmaco





## Medivir is a Nordic research-based pharma company focused on infectious diseases and oncology (2/2)



### **Balanced platform consisting of four cornerstones**

### **Innovation** High risk / high reward

R&D

- Unrivaled expertise in protease inhibitor design and nucleoside/ nucleotide science with focus on infectious diseases and oncology
- Strong pipeline from discovery to development with four internal projects disclosed

Royalties & Milestones

- Two products, Olysio and Xerclear, taken from idea to market and out-licensed to premier big pharma partners
- New deals will add to high-margin cash flow

Innovative Specialty Care Portfolio

- Two innovative specialty care products, Olysio and Adasuve, recently launched in the Nordics and negotiations in process
- Experienced and specialized commercial organization

World class high risk/high reward R&D capabilities with strong current and future cash generation from global milestones/royalties and Nordic commercial operations

### **Established Brands** stable revenue stream

**Nordic Brands** 

14 Rx pharmaceuticals with very stable revenue and earnings generation through efficient organization

### **Financial facts**



CONSOLIDATED INCOME STATEMENT SUMMARY	Q2	Q2	FY
Continuing operations (MSEK)	2014	2013	2013
Net turnover	564.0	40.7	446.1
Gross profit	518.8	23.5	374.3
EBITDA	424.4	-46.9	76.4
EBIT	416.2	-62.0	25.2
Profit/loss before tax	418.4	-62.1	27.7
Profit/loss after tax	327.8	-63.7	16.0
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Net turnover breakdown	Q2	Q2	FY
(MSEK)	2014	2013	2013
Outlicensing and partnership agreements: Non-recurrent payments	•	-	258.5
Pharmaceutical sales	62.9	40.7	176.1
Royalties	501.1	-	11.5
Other services	-	-	-
Total	564.0	40.7	446.1

- Listed on NASDAQ OMX Stockholm since 1996
- Market cap of ~3.5 BSEK / ~500 MUSD
- Shares Outstanding, fully diluted 31,7 M
- Broad institutional shareholder base,
   ~25% EU & US shareholders
- Solid financial position (900 MSEK / 120 MUSD end Q2, 14.)\*
- Transforming to sustainable profitability

<sup>\*</sup>Olysio (Simeprevir) royalties during Q3 of 516 MSEK / 69 MUSD where reported October 14 and are <u>not accounted</u> for in cash position . Medivir Q3 full report will be published November 20.

### Secure current and future value creation through profitable growth



### **Deliver sustainable value creation**

Through world-class R&D productivity, increased commercial focus and operational excellence



### Strengthen R&D pipeline without increasing costs

- Focus on areas of expertise: infectious disease and oncology
- Maintain an average of one project in phase I clinical development



### Capture more pipeline value

Advance projects further (e.g. Phase II)



### Generate diversified revenue from milestones & royalties

• Out-license projects from R&D pipeline



### Become top-tier pharma company in Nordic region, by sales

- Increase commercial focus
- Further expand Innovative Specialty Care Portfolio



### Improve profit margin of Nordic brands

Ensure operational excellence



## Proven ability to create value through R&D efforts, Nordic commercial operation



#### **Innovation**

#### R&D

- Proven track record
- Will continue to be main driver of long term value creation

### **Royalties & Milestones**

 Balance risk/reward through partnerships at costly stages of drug development and global commercialization

### **Innovative Specialty Care Portfolio**

- Significant upside & economies of scale
- Retain Nordic rights for out-licensed products
- In-license products with strong growth potential

## Increasing revenue and earnings with long term stability through

- combination of Nordic sales from multiple products, and
- milestones and royalties from partnerships

Proven ability to discover and develop innovative breakthrough products, partner with premier big pharma companies for late stage development and global distribution, and commercialize own and in-licensed products through strong Nordic platform with economies of scale

## Established brands enhance stability and strength of Nordic commercial operations



#### **Established Brands**

### Nordic Commercial Operations

 Essential supporting functions are common between Nordic Brands & Innovative Specialty Care Portfolio

#### **Nordic Brands**

 Opportunity to further improve margins for the 14 Rx wellknown brands with stable revenue stream



- Significant upside & economies of scale
- Retain Nordic rights for out-licensed products
- In-license products with strong growth potential

Stable revenue stream and economies of scale and scope with Innovative Specialty Care Portfolio

Broad portfolio of established brands with close operational synergies with Medivir's innovative product portfolio



### A focused strategy for value creation based on the four cornerstones



Cornerstones	Strategy
R&D	<ul> <li>I. Project generation and development in R&amp;D</li> <li>Cutting edge competence in protease inhibitor design and nucleoside/nucleotide science with distinct discovery focus on infectious diseases and oncology</li> <li>Focus on true innovation for unmet medical needs to maximize patient benefit and value creation</li> <li>Prudent R&amp;D expense for defined portfolio scope and output (lower spend than 2013/2014)</li> <li>Strategic investments outside of the run-rate to be made through in-licensing, partnerships/collaborations, advancing internal projects into phase II and M&amp;A</li> </ul>
Royalties & Milestones	<ul> <li>II. Partnerships and out-licensing</li> <li>Key component in business model established through proven track record with big pharma</li> <li>Firm commitment to early development in-house before partnership with/out-licensing to global partner to balance risk and optimize value</li> <li>Targeting strong global partners for high quality late stage development and maximum reach in global commercialization</li> </ul>



### **Innovative Specialty Care Portfolio**

**Nordic Brands** 

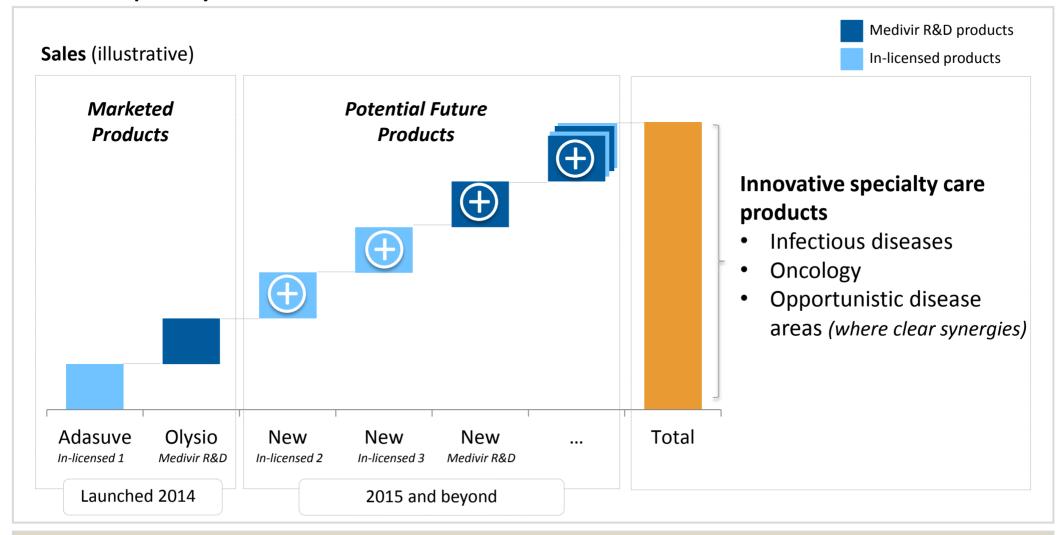
#### III. Commercial operations in the Nordics with focus on innovative products

- Leverage Nordic commercial platform with focus on cost effective utilisation of highly specialised organization by:
  - in-licensing innovative growth products primarily in Infectious diseases and oncology to match R&D focus, but will act opportunistically if synergies can be secured
  - retaining Nordic rights for in-house developed products
- Provides knowledge and insight into entire value chain, including patient benefits, health economics and regulatory matters

## Growing through continuous addition of innovative specialty care pharmaceuticals



### **Innovative Specialty Care Portfolio**



### Key assumption for value creation from in-licensing:

• Net sales per product: Peak sales of approximately 50-150 MSEK within 5 years of launch





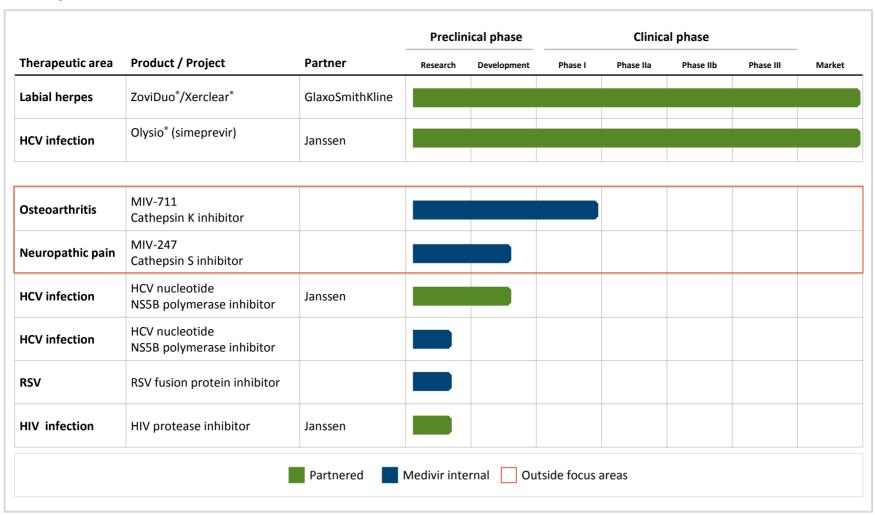
### **Research and Development**

World class science working toward next breakthroughs

## **Extensive partnering and collaboration track record with major pharma**



### **R&D** portfolio



#### Comment

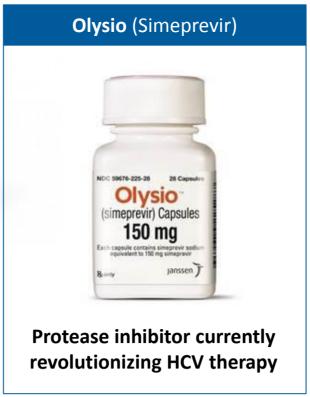
- Strong and diverse early development pipeline
- All future projects will fall within the new focus of infectious disease and oncology
- Ongoing projects include 2 that are outside the new focus

## Medivir has a strong track record of bringing innovative discoveries to market



### **Marketed Products From Own Pipeline Development**





### Medivir's core technology platform:

- Protease inhibitor discovery historically applied to several therapeutic areas including infectious diseases
- Nucleoside/nucleotide science historically applied almost exclusively to infectious diseases

 In the future these technologies will be targeted to specific indications in infectious diseases and oncology

### **Xerclear: Strong payback from innovation**



#### Xerclear / Zoviduo



Nucleoside analogue-based treatment for labial herpes

### **Royalties and Milestones**

- US rights sold to Meda: \$45 million
- GSK RoW OTC rights: €3 million (€1.4 million remaining)
- Royalty agreement on OTC sales: up to 10%
- Royalty payments including Q2 2014: €0,1 million

### **Next steps**

- Estimated OTC market approvals in key EU countries
  - UK: November 2014 / May 2015
  - Spain: September 2015
  - France: September 2016
- Medivir believes that there is still more potential in the brand which the merged GSK/Novartis OTC entity can capitalize on

### Simeprevir: Strong payback from innovation



### Olysio® (simeprevir)



Protease inhibitor currently revolutionizing HCV therapy

### Simeprevir on the global market





USA (OLYSIO®)

Russia (SOVRIAD®)





Australia (OLYSIO®)

### **Royalties and Milestones**

• FTE funding over 3 years: €11 million

Milestones received: €68.5 million

Royalty agreement on Global sales (excl. Nordics): up to 10%

Royalty payments including Q3 2014: €129 million





### **Continued J&J support for OLYSIO®**

### Continued strong commitment from J&J in the HCV area

### J&J's commitment is supported by concrete actions

### Clinical trial programme

- Two phase III studies, OPTIMIST 1 and 2, evaluating treatment of genotype 1 HCV-infected patients with simeprevir and sofosbuvir, are well under way.
- Yet another phase II IFN-free triple combination of simeprevir, sofosbuvir, and daclatasvir (IMPACT) in decompensated cirrhotic HCV patients has been announced
- One phase II study of an IFN-free triple combination of the three compounds simeprevir, IDX719, and TMC055 (HELIX-2)
- One phase II study including another IFN-free triple combination; simeprevir, JNJ-56914845, and TMC055

M&A

 On November 7, J&J announced the closing of the acquisition of Alios BioPharma that has two anti-hepatitis C virus (HCV) nucleotides in development which, if successful, would complement simeprevir and secures J&J continues to be a leader in the disease area

Medivir believes that IFN-free treatments will be dominating, with many more options available for physicians and patients than today, but there are on-going studies including IFN and RBV that are worth highlighting:

- 12 weeks full stop single-arm phase III study in treatment naïve GT1 and GT4 patients
- China: efficacy, safety & tolerability and pharmacokinetics in treatment naive GT1 HCV



World class science working toward next breakthroughs

## Flexible and productive R&D organization to ensure strong early development pipeline



### **R&D** organization and capabilities

### **Description**

World class discovery R&D organization

- Core competence developed and refined over 26 years from idea through early clinical development
- Infrastructure to support multiple projects in parallel

Prudent use of resources

- Efficient organization and infrastructure enables us to sustain internal pipeline, with future running annual expenditure below 2013-2014 levels
  - 2-3 LO projects at steady state, expected to deliver on average 1 well-differentiated candidate drug/yr (Sufficient lead identification capacity to sustain the targeted LO efforts)
  - Ambition to maintain an average of one project in phase I clinical development (Development capacity to support advancement of all internal candidates through phase 1)

Flexibility to expand / accelerate development

- Flexibly organized to enable acquisition or in-licensing of projects capable of expanding and accelerating pipeline development and value creation:
  - Strengthening of the early development pipeline (LO → Phase I)
  - Enabling expansion into oncology

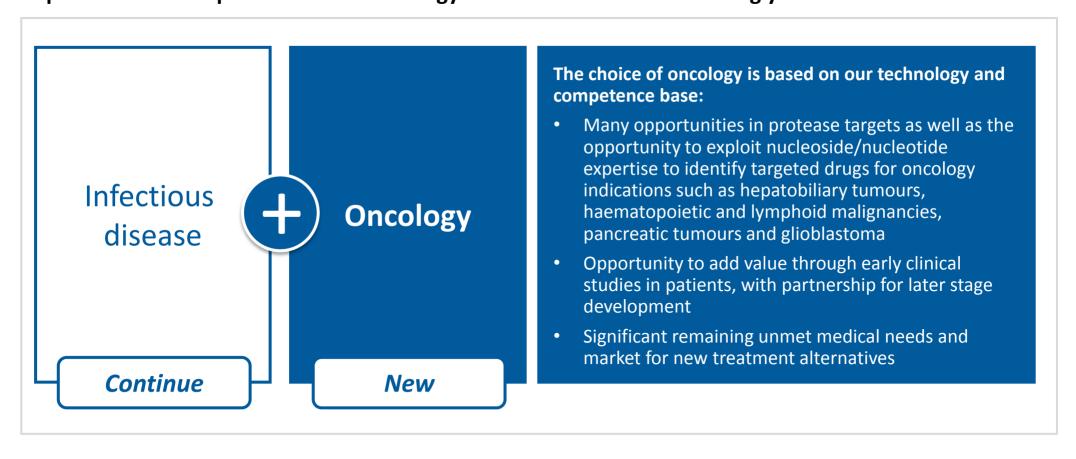
Advancement of internal projects in the value chain

- Medivir will advance high-value projects into phase IIa (PoC) provided there is a strong business rationale
  - Progress into phase II will be based on robust financial rationale
  - Internal medical expertise available for designing Phase II clinical studies and managing CRO during clinical operations

### Leverage our technology platform to capture opportunities in new focus areas for future value creation



Alongside continued focus on infectious diseases, we will direct the company's technological expertise towards specific areas in oncology over the course of the coming years





### **MIV-711**

a once daily potent and selective **cathepsin K protease inhibitor** in clinical development for the treatment of **osteoarthritis** 



### Osteoarthritis (OA) – a leading cause of chronic disability

### **Overview**

- Progressive disorder characterized by degeneration of cartilage and subchondral bone in the joints
- Most prevalent joint disease with up to 40% over 65 suffering from knee or hip OA
- Current treatments are inadequate focusing on symptom relief e.g. physiotherapeutic exercise, intra-articular corticosteroids or hyaluronic acid and analgesics/anti-inflammatory agents (NSAIDs)
- Large burden to society: 15 million QALYs\* lost annually in US only, comparable to cardiovascular disease and cancer

### Key unmet needs

### Suspend disease progression and relieve pain

- Prevent degradation of subchondral bone recently recognized as a key target for OA
- Prevent degradation of cartilage
- Prevent the pain associated with the disease

A disease-modifying OA drug (DMOAD) meeting these unmet needs has great market potential based on large and growing patient population

### MIV-711 – has the potential to become the first disease-modifying osteoarthritis treatment on the market



#### **Mode of Action**

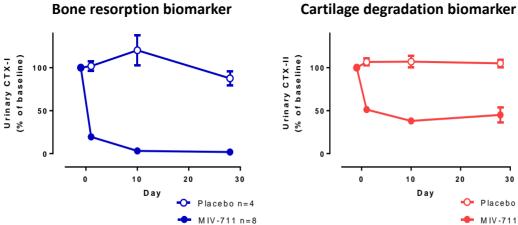
### The protease Cathepsin K degrades both bone and cartilage collagen: inhibition of this enzyme is expected to have joint protective effects in humans

 Other bone-acting agents have demonstrated beneficial effects on human osteoarthritis (OA) disease progression, pain and function

#### MIV-711 Profile

### A Cathepsin K inhibitor with strong profile

- Demonstrated joint protective effects in established preclinical OA models
- Generally safe and well tolerated up to 28 days in humans
- Markedly reduced bone and cartilage degradation in phase I as demonstrated by biomarkers:



### **Next steps**

Placebo n=4

- **Toxicology studies** (6 months) recently initiated to enable start of Phase IIa study in osteoarthritis patients late 2015
- Innovative biomarker driven development path

### MIV-711 – a large and growing market opportunity



### **Market opportunity**

- 250 million people worldwide estimated to suffer from knee OA in 2012 (Nat. Rev. Rheumatol., 2014)
- Prevalence of OA is increasing due to aging population and obesity epidemic



 A DMOAD\* cost of approx. 3,000 USD/Y has been estimated to satisfy cost-effectiveness criteria based on suspended disease progression and pain relief (Losina et al 2014)

### **Target population / indication**

MIV-711 - targeted towards adult patients with moderate osteoarthritis in weight bearing joints (>2 millions in US only) to:

- delay disease progression
- reduce need of pain relief and
- improve function

### **Summary**

- Very large and attractive market opportunity
- Every 10% of the target population on the US market alone represents a potential of 600 MUSD\*\* in annual sales
- Opportunity also in other bone-related diseases

<sup>\*</sup> DMOAD: Disease-modifying Osteoarthritis drug

<sup>\*\* 10%</sup> market share represents 200,000 patients multiplied by an annual treatment cost of 3,000 USD/Year



### **MIV-247**

a potent, selective cathepsin S protease inhibitor in non-clinical development as an oral therapy for neuropathic pain

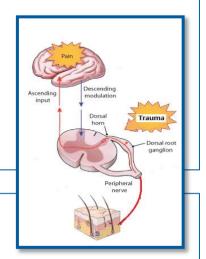
## Neuropathic Pain (NP) – a large market opportunity for novel, safe and efficacious treatments



#### Overview

- Affects ~30 M people in the 7 major markets
- Caused by a trauma or disease affecting the nervous system such as diabetes, shingles, cancer or chronic lower back pain
- Limited efficacy and poor side effect profiles of current treatments, including anticonvulsants (e.g. pregabalin & gabapentin) and antidepressants, (e.g. amytriptyline)

• Overall sales in NP market 2012: 6 BUSD (pregabalin: 1.8 BUSD, Lidocaine 5% patch: 0.7 BUSD and Duloxetine 0.6 BUSD + generic opioids and/or NSAIDs)



### Key unmet needs

More efficacious Neuropathic Pain specific drugs with less side-effects and rapid onset

A novel Neuropathic Pain treatment meeting these needs will have a market opportunity of > 1BUSD in annual sales

Source: Decision Resources

## MIV-247 – A new targeted Mode of Action in an underserved pain market



#### Mode of Action

Cathepsin S expression is increased in the nervous system post nerve injury where it may release of inflammatory mediators leading to pain

#### MIV-247 Profile

### A potent cathepsin S inhibitor for oral treatment of NP

- Efficacious as monotherapy in preclinical neuropathic pain models
- Markedly enhanced efficacy in combination with pregabalin or gabapentin compared to either drug alone
- No CNS side effects at highest efficacious dose

### **Next Steps**

- Preclinical IND preparatory safety package recently initiated
- Start of clinical Phase I program planned in 2Q 2015

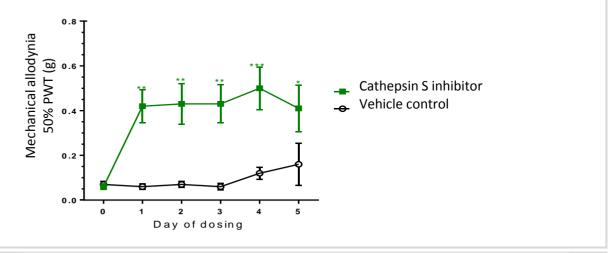
Source: Datamonitor & Decision Resources

## Medivir's cathepsin S inhibitors – efficacious as monotherapy and enhanced effects with gabapentin in a model of Neuropathic Pain



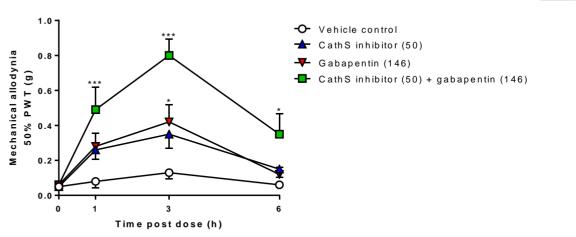
### **Monotherapy**

Fast and sustained effects of cathepsin S inhibition in a model of neuropathic pain



### **Combination therapy**

Markedly enhanced effects with a cathepsin S inhibitor and gabapentin at *minimal* effective doses



### Data support therapeutic value in a broad Neuropathic Pain patient population

- As first line monotherapy
- As an add-on to current SoC with potential to increase efficacy while decreasing side effect by lowered doses of the companion drug



### **HCV Nucleotide**

a wholly-owned uridine protide with potent activity against all genotypes of hepatitis C virus for use **as** part of all-oral treatment regimens

### A cornerstone of HCV therapy



### Hepatitis C Overview

- HCV therapy being revolutionized by all-oral interferon free regimens
- Future therapy expected to be 3-drug combinations with cross-genotype activity to achieve shortened durations of therapy
- Nucleotides will be the cornerstone of such combinations because of their high level of antiviral activity, cross-genotype activity and high barrier to resistance

### Medivir's project

- Leveraging of nucleoside expertise to pursue high value nucleotide compounds
- Current effort focused on novel uridine-based series
- Medivir protide's preclinical profile:
  - Potent cross-genotype antiviral activity
  - Attractive pharmacokinetic and resistance profile



### **Market Opportunity**

- Compound will be competition for Sovaldi™ and Idenix-21437
- Large potential for nucleotides overall but actual potential for Medivir's nucleotide is dependent on the competitive landscape at launch

### **Next Steps**

 Profiling of the lead clinical candidates in progress, with potential decision on continued program by year end



### **RSV Fusion Inhibitor**

Lead optimization project focused on delivery of a best-in-class orally administered antiviral drug for the treatment and prophylaxis of RSV infection

## Respiratory Syncytial Virus (RSV) Infection – Significant market potential in an under-recognized disease



### RSV infection Overview

- · Seasonal outbreaks of respiratory tract infections of children and adults
  - Mild respiratory illnesses through life-threatening bronchiolitis and pneumonia
- RSV causes repeated infections throughout life but especially dangerous in:
  - Infants, especially premature babies and those with lung/heart disease
  - The elderly, especially those with cardiovascular morbidities
  - Immunocompromised patients
- Principal RSV drug is restricted to prophylactic use exclusively in the highest risk infant population

### Medivir's project

- Clinically validated target
- Opportunity to exploit our proven strengths in antiviral discovery & early development
- In-licensing the RSV project represented a rapid and cost-effective opportunity to acquire a LO phase project into the R&D pipeline
  - Most recent example of Medivir's strategic intent to enhance its R&D pipeline with highvalue, commercial opportunities

### **Market Opportunity**

 Market potential (based on health-care utilization by young children and elderly patients infected by RSV) is estimated to be 500 MUSD in annual sales

### **Next Steps**

 Currently in lead optimization phase, with decision on CD nomination expected in 2016



### **Prioritized Opportunities for future R&D investments**

### Strategic investments will focus on:

- 1) Bringing our internal projects to next value inflection point based on robust financial rationale, e.g.
  - MIV-711 phase IIa proof-of concept in osteoarthritis patients
  - MIV-247 phase IIa proof of concept study in neuropathic pain patients
- 2) In-licensing of oncology project(s)
  - Accelerate oncology pipeline using our internal competence in key areas
- 3) Intensify partnering and collaboration
  - Target access to external innovation and funding to accelerate portfolio development
- 4) Exploring targeted M&A
  - Strategic opportunities to expand the oncology portfolio

All opportunities evaluated as business cases







Strong outlook for coming years
Summary

## Targeted investments to enhance value, while also distributing cash to shareholders (1/2)



Financial strength enables Medivir to benefit shareholders and ensure investment in future value creation

Optimization of capital structure

 Strong financial position allows for cash distribution to shareholders and investments in future value creation

Prudent use of resources

- Corporate running costs will be tightly-controlled and reduced compared to 2014
- Investments will be made in well-defined areas:
  - to increase output from R&D
  - to maximize the value of current projects
  - to accelerate revenue growth in Innovative Specialty Care Portfolio

## Targeted investments to enhance value of R&D portfolio and improve revenue growth, while also distributing cash to shareholders (2/2)





### Dedication and focus from everyone at Medivir on building and visualizing the value of each cornerstone



- R&D expected to:
  - Focus on specific areas in oncology in addition to infectious diseases
  - Strengthen early development pipeline
  - Advance current projects to out-licensing while balancing risk and value maximization

Royalties & Milestones

- Royalties from simeprevir expected to decline vs. 2014, but to remain a significant contribution to Medivir both medium and long term
- Income from new out-licensed projects expected to provide growth and risk diversification

Innovative
Specialty Care
Portfolio

 Sales expected to grow in the medium and long term on the launch of new products, after initial decline in Olysio sales in 2015

**Nordic Brands** 

• Expected to provide stable revenues and become increasingly profitable as margins improve