MEDIVIR Q2 CALL
28 AUGUST 2019
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### Financial summary

#### Summary of the Group’s figures

<table>
<thead>
<tr>
<th>(SEK m)</th>
<th>2019</th>
<th>2018</th>
<th>2019</th>
<th>2018</th>
<th>2018</th>
</tr>
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<tbody>
<tr>
<td>Net turnover</td>
<td>3,7</td>
<td>2,8</td>
<td>5,7</td>
<td>7,3</td>
<td>23,9</td>
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<tr>
<td>Profit/loss before tax</td>
<td>-12,4</td>
<td>-92,7</td>
<td>-68,3</td>
<td>-164,7</td>
<td>-350,7</td>
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<td>Cash and cash equivalents at period end</td>
<td>191,9</td>
<td>438,6</td>
<td>191,9</td>
<td>438,6</td>
<td>286,3</td>
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- Net turnover for Q2 2019 was SEK 4 million and for H1 SEK 6 million
- Loss of the quarter Q2 was SEK -12 million and for H1 SEK -68 million
- Cash position as of June 30, 2019: SEK 192 million
- Market cap as of August 27, 2019: approximately SEK 592 million
Highlights

- Clinical Development focus on oncology
  - Birinapant/Keytruda®: 15 patients recruited to the phase II study in colorectal cancer. Interim analysis planned for Q4 2019.
  - MIV-818: Phase Ia in liver cancer patients evaluated in Q2 2019
- Business Development focus on phase III ready remetinostat and MIV-711
- Staff reduced to 14 FTE
- Reorganization will lead to fixed cost reduction by about two-thirds
- New organization is strong and cost-effective
Experienced leadership

Uli Hacksell, PhD; CEO
Uppsala U, Astra, ACADIA

Magnus Christensen, MBA; CFO
O’Learys Trademark, ICA Sverige, HKScan

Christina Herder, PhD; COO
Pharmacia, Biovitrum

Linda Basse, MD; PhD; CMO
Abbott, Topo Target, Genmab, Zealand

Rikard Höse, MD; Med Dir.
Karolinska U Hospital, Novartis

Linda Palmér, Sr Dir Clin Ops
Pfizer

Fredrik Öberg, PhD; CSO
Uppsala U
# Broad and robust pipeline

<table>
<thead>
<tr>
<th>PROJECT &amp; MECHANISM</th>
<th>DISEASE AREA</th>
<th>RESEARCH</th>
<th>PRECLINICAL</th>
<th>PHASE I</th>
<th>PHASE II</th>
<th>PHASE III</th>
<th>EXCLUSIVITY</th>
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<tbody>
<tr>
<td>Remetinostat HDAC INHIBITOR (TOPICAL)</td>
<td>Cutaneous T-cell lymphoma (MF)</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
<td>IP: 2034</td>
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<tr>
<td></td>
<td>Basal cell carcinoma&lt;sup&gt;1&lt;/sup&gt;</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Birinapant SMAC MIMETIC (INTRAVENOUS)</td>
<td>Colorectal cancer (combo with Keytruda&lt;sup&gt;®&lt;/sup&gt;)</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
<td>IP: 2034</td>
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<tr>
<td>MIV-818 NUCLEOTIDE DNA POLYMERASE INHIBITOR (ORAL)</td>
<td>Hepatocellular carcinoma</td>
<td></td>
<td></td>
<td></td>
<td></td>
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<tr>
<td>MIV-828 NUCLEOTIDE DNA POLYMERASE INHIBITOR (INTRAVENOUS)</td>
<td>Hematological malignancies (acute myeloid leukemia)</td>
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<tr>
<td>MIV-711 CATHEPSIN K INHIBITOR (ORAL)</td>
<td>Osteoarthritis</td>
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<td></td>
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<sup>1</sup> Investigator sponsored study at Stanford U.

<table>
<thead>
<tr>
<th>Status</th>
<th>Ongoing</th>
<th>Completed</th>
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*Slide 6*
Remetinostat for early-stage cutaneous T-cell lymphoma
MF-CTCL: orphan blood cancer indication

Cutaneous T-cell lymphoma (CTCL) affects lymphocytes (cells belonging to the immune defense system) located in the skin and typically has a chronic course.

- CTCL is a rare form of non-Hodgkin lymphoma primarily present in the skin. Mycosis fungoides (MF) is the most common form of CTCL
- Annual new cases: US ~ 2,000; EU ~ 3,000; Sweden ~ 25
- Five-year survival: ~ 85%; more than 16,000 US patients live with MF-CTCL
- Skin lesions and severe itching are common and affect patients quality of life
- Early stage disease lasts for long periods and requires well tolerated therapy
- Available treatments, such as Vorinostat and other systemic HDAC inhibitors, bexarotene, and Valchlor, have severe side effects
Remetinostat: for treatment of early stage MF-CTCL

- Remetinostat is a histone deacetylase (HDAC) inhibitor
- Remetinostat’s unique chemistry and topical formulation provides for activity in skin and rapid degradation in blood
- Approved HDAC inhibitors not used in early-stage MF-CTCL patients
- US orphan drug designation
Remetinostat: clinical proof-of-concept phase II MF-CTCL study

Twelve months phase II data shows reduction in both lesions and severe itch

<table>
<thead>
<tr>
<th>Dose</th>
<th>1% 1x/day n=20</th>
<th>0.5% 2x/day n=20</th>
<th>1% 2x/day n=20</th>
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</thead>
<tbody>
<tr>
<td>Lesion responses(^1)</td>
<td>20%</td>
<td>25%</td>
<td>40%</td>
</tr>
<tr>
<td>Patients with clinically significant pruritus</td>
<td>(40%) n=8/20</td>
<td>(30%) n=6/20</td>
<td>(50%) n=10/20</td>
</tr>
<tr>
<td>Pruritus responses</td>
<td>38%</td>
<td>50%</td>
<td>80%</td>
</tr>
</tbody>
</table>

Well tolerated:
- No HDAC inhibitor-associated systemic adverse events
- Median time on treatment: 336 days (1% 2x/day dose)

\(^1\) Confirmed responses based on CAILS, the Composite Assessment of Index Lesion Severity
Remetinostat: next steps

- Medivir has defined a phase III design based on the requirements clarified by the FDA
- One phase III study expected to be sufficient for FDA
- Medivir seeks to identify a business partner for the further development of remetinostat
Remetinostat: interim phase II BCC data presented at SID 2019*

Basal cell carcinoma

• The most common form of cancer in humans occurring in the skin
• Surgery is standard of care, but there is a need for efficacious and safe treatments when surgery is impractical, e.g. multiple lesions and/or difficult treatment sites

Interim phase II data

• Fifteen patients recruited in open-label study
• Treatment: remetinostat gel 1% (with occlusion) 3 times/day for six weeks
• ORR (≥ 30% in longest diameter): 64%
• 43% of tumors fully cleared
• No systemic toxicities
• Grade 2 reversible eczematous reaction in 71% of patients

* Urman et al., An open label phase 2 clinical trial of topical remetinostat for basal cell carcinoma
Birinapant: Uniquely potent against selected solid tumors
Colorectal cancer - Large unmet medical need

Many patients with colorectal cancer have limited treatment options and are in need of new effective medicines to extend life.

The immuno-oncology medicine Keytruda® on its own is not sufficiently effective in treatment of MSS colorectal cancer.

Colorectal cancer indication (CRC)
  - The second most common cancer in women and the third in men
  - Estimated new cases 2018: US: ~ 140,000; EU: ~ 490,000; Sweden: ~ 6,200
  - Five-year survival: 14% when metastatic
Birinapant may benefit patients with inadequate response to immuno-oncology therapies

- Birinapant, a SMAC mimetic, enables tumor cell death and augments the immune system
- Great potential to improve treatment of cancers when combined with immuno-therapy
- Ongoing collaboration with Merck for a phase I/II study in solid tumors
  - Joint development committee oversees the study
  - Keytruda® provided at no cost by Merck
  - Medivir retains full global rights to birinapant and data
Birinapant/Keytruda® combination - phase II study ongoing

Dose escalation study completed; December 2018

- Nineteen patients with solid tumors and without further treatment options were enrolled

Phase I: Sequential group dose-escalation to determine safety and tolerability and establish the recommended phase II dose.

Phase II: Safety, tolerability and efficacy of the recommended dose of birinapant in defined disease cohorts.

Phase I (n= 19 pts)
Interpatient dose escalation (3+3 design)

RP2D

Phase II expansion, n≤30 pts per indication
Birinapant/Keytruda® combination, outcome and the future

Dose escalation study:
Combination of birinapant and Keytruda® is safe and well tolerated

Two patients are still on treatment:
- One MSS CRC patient has been on treatment for over 80 weeks and has achieved PR
- One osteosarcoma patient has been on treatment for over 30 weeks and has achieved SD

Two patients achieved a PR and seven patients achieved SD

Phase II dose selected at 22 mg/m²

Phase II dose-expansion study is ongoing in MSS CRC patients
- First patient dosed December 2018
- Fifteen patients recruited
- Futility analysis in Q4, 2019
MIV-818: Nucleotide prodrug for the treatment of liver cancer
Introduction

**HCC is the third leading cause of cancer-related deaths worldwide**
- Estimated new cases 2018: Asia: ~ 610,000; US: ~ 42,000; EU: ~ 82,000
- Orphan disease in Western markets, but one of fastest growing and most deadly cancers in US
- High incidence in Asia including China - Hepatitis B & C very common
- Five-year survival: 18%
- Genetically heterogeneous leading to limited effect of molecularly targeted therapies

**MIV-818 for treatment of liver cancer**
- MIV-818 is a proprietary new chemical entity discovered at Medivir
- MIV-818 is being developed as a new treatment for HCC and other liver cancers as a stand alone treatment or in combination with standard of care

**Patients with advanced liver cancer are in need of new therapies**
Mechanism of Action

Chain-terminating inhibition of DNA synthesis

- **MIV-818** is an orally administered nucleotide prodrug of the active metabolite **troxacitabine triphosphate** (TRX-TP).

- When incorporated into DNA, TRX-TP causes double strand DNA breaks and cell death.

- Troxacitabine progressed to Phase 2/3, with clinical responses observed in several cancers, but development halted due to the narrow therapeutic window.

Liver targeting to deliver high levels of the active metabolite to the liver while minimizing exposure elsewhere

- **MIV-818** has been designed to minimize systemic exposure and limit the toxicity of troxacitabine by primarily targeting the liver.

- This prodrug technology has been clinically proven to deliver high liver levels of nucleotides in patients with compensated cirrhosis\(^1\)

Phase I - study design

Phase I

Phase Ia
Intrapatien dose escalation

Phase Ib
Interpatient dose escalation

Phase Ia data evaluation and decision
to move into phase Ib 3+3
interpatient dose escalation based
on SRC recommendation

Phase I data evaluation and
decision about the
recommended dose for
phase II (RP2D) based on SRC
recommendation
Study design and patient population

- The primary aim of the phase Ia study is to evaluate the safety and tolerability of MIV-818.
- In addition, exploratory objectives include pharmacokinetics and biomarkers of activity.
- The patients included have advanced liver cancer i.e. hepatocellular carcinoma, intrahepatic cholangiocarcinoma and liver metastatic disease.
- The patients have been treated with escalating doses of orally administered MIV-818.
MIV-818 induces DNA-damage response in liver tumour tissue

Clear evidence of pH2AX induction (brown nuclear stain) resulting from DNA-damage in tumour but not normal liver tissue.

Data from Patient 2

Data from Patient 4
MIV-818 shows activity in hypoxic regions of liver tumours

• Equal frequency of pH2AX positive nuclei observed in regions of high membrane Glucose transporter 1 (Glut1) staining
• Indicates that MIV-818 reaches hypoxic areas and induces DNA-damage (common limitation for chemotherapy)

Glut1 membrane expression (hypoxia)

Data from Patient 2
Data from Patient 4
Phase Ia – summary preliminary data

- MIV-818 has been well tolerated. Lowering of blood counts have been observed in two patients.
- Clear signs of effect, measured as DNA damage, observed in liver biopsies from tumor tissue in MIV-818 treated patients. Normal liver tissue does not appear to have been affected.
- This tumor selective effect was observed at low levels of MIV-818 in plasma and is an early indication that MIV-818 has the intended liver-directed effect.
Next steps

- The results from the first six patients are very positive
- Medivir has decided to initiate the phase Ib part of the MIV-818 study as soon as the independent safety committee has given its recommendation on an appropriate starting dose.
- A few more patients will be recruited in phase Ia to ensure that the dose-selection for phase Ib is optimal
MIV-711: Cathepsin K inhibitor with FDA fast track status
Osteoarthritis (OA): the most common form of joint disease

- Affects >30m adults in the US, and ~240m worldwide
- Prevalence increasing due to aging population and obesity epidemic
- Current treatments focus only on pain relief
- Large unmet medical need for a disease-modifying drug (DMOAD) with potential to slow, halt or reverse the progression of OA

Cathepsin K protease is involved in the breakdown of collagen I in both bone and collagen II in cartilage.
MIV-711: positive effects on joint structure and signals of benefit on clinical symptoms

- Study design (MIV-711-201): 3 arms, 26 weeks treatment
- Study design (MIV-711-202): 200 mg MIV-711, 26 additional weeks

MIV-711-201: Change from baseline vs week 26

<table>
<thead>
<tr>
<th></th>
<th>PBO n=80</th>
<th>n=80 100 mg MIV-711 QD</th>
<th>n=80 200 mg MIV-711 QD</th>
</tr>
</thead>
<tbody>
<tr>
<td>Femur bone area (mm²)</td>
<td>23.2</td>
<td>8.1</td>
<td>8.2</td>
</tr>
<tr>
<td>Cartilage thickness (mm)</td>
<td>-0.066</td>
<td>0.008</td>
<td>-0.017</td>
</tr>
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</table>

- A trend consistently favoring MIV-711 arms in all predefined analyses of clinical outcomes, e.g. knee pain and knee function
- Safety and tolerability profile supporting advancement of MIV-711 as a disease-modifying OA drug candidate
- New US FDA guidelines in OA may enable pathway for accelerated regulatory approval
Value Drivers
Near term value inflection points

- MIV-818: Phase Ib study initiated – Q4 2019
- Birinapant/Keytruda®: futility analysis completed – Q4 2019