HEL SINN GROUP 2014

Jefferies Healthcare Conference
New York, June 5th, 2014
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Helsinn – Building Quality Cancer Care Together

- A world-leading company focused on cancer supportive care
- Our goal is to build a quality portfolio of innovative cancer-care therapies through licensing, development and connections within the cancer-care community
  - industry-leading commercialization success rate of 68%
- A comprehensive portfolio of therapies treating several indications including: chemotherapy-induced nausea and vomiting, pain and inflammation and gastroenterology
- Our products are sold in more than 90 countries worldwide via more than 65 partners
- Growing global footprint, with state-of-the-art R&D facility in Biasca, Switzerland, a world-class production plant in Dublin, Ireland and a growing presence in both China and the US
- Privately held, founded in 1976 by Dr. Gabriele Braglia, headquartered in Lugano, Switzerland, now more than 560 employees
Helsinn’s Senior Executive Team

Riccardo Braglia
Group Chief Executive Officer,
Board Member & US Chairman

Giorgio Calderari
Group General Manager &
Chief Operating Officer &
US Board Member
29 years

Luigi Caletti
Group Chief Financial Officer,
Board Member &
US Board Member
25 years

Waldo Mossi
Local General Manager,
Chemical Business,
Helsinn Advanced Synthesis,
Switzerland
20 years

Padraig Somers
Local General Manager,
Pharma Business,
Helsinn Birex Pharmaceuticals,
Ireland
22 years

William Mann
Local President & CEO,
US Board Member
Pharma Business, Helsinn Therapeutics
(U.S.), Inc
5 years

Andrea Meoli
Corporate Chief
Commercial Officer
12 years

Matteo Missaglia
Senior Director,
General Counsel,
Corporate Legal Affairs
24 years

Sergio Cantoreggi
Corporate Chief Scientific
Officer
14 years

Roberto De Ponti
Senior Director,
Head of Corporate
Business Development
6 years

Daniele Bonadeo
Senior Director,
Head of Corporate
Technical Affairs
16 years
Helsinn’s International Business Operations

Helsinn Group’s US operations:
Helsinn Therapeutics US Inc.,
Bridgewater, NJ, USA

Helsinn Group’s development, manufacturing and supply chain platform for finished drug products:
Helsinn Birex Pharmaceuticals Ltd.,
Dublin, Ireland

Helsinn Group’s Global Headquarters
Helsinn Healthcare SA,
Lugano, Switzerland

Helsinn Group’s development & manufacturing facility for Active Pharmaceutical Ingredients:
Helsinn Advanced Synthesis SA,
Biasca, Switzerland

Helsinn’s representative office: Beijing, China
Helsinn’s Business Approach: Integrated Licensing

- Value-added support
- Our mission is to help people with cancer get the most out of every day
- So to ensure patients receive the best possible treatment, our partners receive our best possible support
- Activities and programs are carried out on an individual basis, based on specific partners’ needs and objectives throughout all the life cycle of the products
Helsinn’s Partners Worldwide

- Aché
- Alliance Pharma
- Andromaco
- Angelini
- Aspen Pharma Pty
- Atco Lab.
- Azevedos
- Biotoscana
- Boehringer Ingelheim
- Bonifar D.O.O.
- Chugai Pharma Marketing
- CJ Corp.
- CSC
- Dara Biosciences
- Eisai
- Especificos Stendhal
- Eurofarma
- Fannin Ltd.
- Fidia
- Galenica
- GEN ilaç
- Grünenthal
- GSK
- Harvestor Trading Company
- Hemas
- Holling Bio Pharma
- Indochina Healthcare
- Italfarmaco
- JW Pharmaceuticals
- Kampar
- Lab. Ergo-Maroc
- Lab. Rubio
- Lab. Silesia
- Lee’s Pharma
- Lusomedicamenta
- Mantecorp
- Merck & Co.
- Mundipharma
- Naturalia
- Norgine
- Novamed
- Noventure
- Nycomed
- Onko Koçsel Ilac
- Ono Pharmaceutical
- Pfizer
- Pharmasolutions
- Pharmaswiss
- P.T. Kalbe Farma
- Quality Pharma Eirl
- Rafa Laboratories
- Restore Medica
- Riemser Arzneimittel AG
- Riso Pharma Tech
- Robapharm
- Roche
- Sinclair IS Pharma
- Sobi
- Specialised Therapeutics
- Sulkaj
- Taiho
- Taiwan Major Chem. & Co.
- Thorne Research
- Tramedico
- Treasure Mountain
- Twinpharma BV
- UCB Pharma
- Vifor Pharma
- Vifranco Pharma Co.
- Widepharma
Financials
Helsinn’s 2013 Financial Highlights

REVENUE $348 MILLION
5.45% CAGR* IN PAST 5 YEARS

REVENUE BREAKDOWN BY REGION

- US: 49%
- EU: 29%
- Japan: 11%
- RoW: 8%

REVENUE BREAKDOWN BY THERAPEUTIC AREA

- Palonosetron: 82%
- Netupitant: 8%
- Nimesulide: 8%
- Others (Chemicals and minor products): 8%
- Chemical: 2%
- others: 2%
- Cancer care: 11%
- Gastroenterology: 11%
- Others (Chemicals and minor products): 11%
- Pain and Inflammation: 11%
- Others: 11%

* Compounded Annual Growth Rate (incl. HPM)
Historic Financial Charts
Revenues, Profitability, R&D figures and number of employees

Net Revenues in Million USD

EBITDA in Million USD

R&D Expenses in Million USD

Headcounts at YE
Helsinn Current Portfolio and Expected Sales in USD

YEAR OF PEAK SALES

- Axol 252 M
- Daxibe 0.4 M
- Gelclair 8.7 M
- Nepa 214.2 M
- Anam 426.1 M
- Tide 134.5 M

Development Stage

- Approved
- Oxap 1.1 M
- Nime 26.6 M
- Klean Prep 7.0 M
Our Cancer Supportive Care Focus
Cancer Supportive Care Focus Areas:

- Chemotherapy-induced nausea and vomiting
- Cachexia and Anorexia
- Oral Mucositis
- Hand and Foot Syndrome
- Cancer induced pain
- Diarrhea
- Pre colonoscopy treatment
- Medical Devices
- Alternative Therapies
Cancer Supportive Care Space

<table>
<thead>
<tr>
<th>Category</th>
<th>Emerging Market</th>
<th>Underserved Market</th>
<th>Well-served Market</th>
</tr>
</thead>
<tbody>
<tr>
<td>Example Therapeutic Areas</td>
<td>- Hand/Foot Syndrome&lt;br&gt;- Chemotherapy/Radiotherapy-associated Dermatitis&lt;br&gt;- Cachexia – Sarcopenia&lt;br&gt;- Fatigue&lt;br&gt;- Chemotherapy-induced Diarrhea&lt;br&gt;- Lymphoedema&lt;br&gt;- Brain Edema&lt;br&gt;- Polypharmacy/Combinations</td>
<td>- Oral Mucositis&lt;br&gt;- Ototoxicity&lt;br&gt;- Cognitive disorders / depression&lt;br&gt;- Chemotherapy-induced Neuropathy&lt;br&gt;- Cancer Pain&lt;br&gt;- Chemotherapy-induced Alopecia&lt;br&gt;- Opioid-induced Constipation</td>
<td>- Chemotherapy-induced Nausea &amp; Vomiting&lt;br&gt;- Anemia&lt;br&gt;- Neutropenia&lt;br&gt;- Thrombocytopenia&lt;br&gt;- Cardio protection&lt;br&gt;- Osteoporosis&lt;br&gt;- Sexual disfunction</td>
</tr>
<tr>
<td>Growing the Portfolio</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Competitive Landscape</td>
<td>- Pipeline is sparse&lt;br&gt;- Targeted therapeutics are expected to be used more frequently and/or over a longer time period, often in conjunction with traditional chemotherapy</td>
<td>- Few commercially available therapeutics and relatively small pipeline</td>
<td>- Highly competitive – several marketed products and robust pipeline&lt;br&gt;- Second-generation products primarily based on new delivery approaches</td>
</tr>
</tbody>
</table>
Helsinn’s Commercialized Products

ALOXI® ONICIT® PALOXI®
Chemotherapy-Induced Nausea and Vomiting (CINV)
Post-Operative Nausea and Vomiting (PONV)

GELCLAIR®
Oral mucositis

KLEAN-PREP®
Bowel preparation prior to Colonoscopy and Surgery

NIMESULIDE
(eg. Aulin® Mesulid® Nimed® Nexen®)
Pain and Inflammation

OXAPROZIN
(Duraprox® Walix®)
Pain and Inflammation
Palonosetron in CINV

CANCER CARE

- Palonosetron (palonosetron hydrochloride) is the market-leading treatment for chemotherapy-induced nausea and vomiting (CINV) in people with cancer and post operative nausea and vomiting (PONV)
- **Compound sales since launch of $4.1 billion; Top seller in more than 50 countries worldwide as Aloxi®, Onicit®, Paloxi®**
- Palonosetron is a second generation 5-HT3 receptor antagonist
- Long half-life of 40 hours, binds more tightly and in a different way to the receptor as compared to other available drugs of the same class, providing greater efficacy

Read more: www.aloxi.net
GELCLAIR

ORAL CANCER CARE

- Gelclair® is a bioadherent oral gel for mucositis relief (Medical Device approved in USA in 2001).
- Used as a mouthwash – it forms a protective film by adhering to the mucosa of the oropharyngeal cavity.
- Gelclair® provides pain relief and helps preventing irritations of the mouth caused by radiotherapy and chemotherapy.

**GELCLAIR coats exposed nerve ending of the mucosa throughout phases of oral mucositis**

Read more: www.gelclair.net
Klean Prep

GASTROENTEROLOGY

- Successful PEG based specialty product for gastrointestinal lavage and preparation prior to diagnostic examination or surgery.
- Proven efficacy and safety over 25 years of use in more than 45 million procedures worldwide
- The main ingredient in Klean-Prep® is PEG 3350 which acts as laxative with various salts added to maintain the body’s electrolyte balance

Read more: www.klean-prep.net
Nimesulide

PAIN AND INFLAMMATION

- A non-steroidal anti-inflammatory drug (NSAID) with an over 25-year history in the treatment of acute pain and primary dysmenorrhea
- Licensed in over 50 countries

Read more: www.nimesulide.com
Development Pipeline
# Helsinn’s Development Pipeline

## Cancer Care

<table>
<thead>
<tr>
<th>Drug</th>
<th>Condition</th>
<th>Status</th>
</tr>
</thead>
<tbody>
<tr>
<td>Netupitant-Palonosetron</td>
<td>Chemotherapy-Induced Nausea and Vomiting</td>
<td>RESEARCH PRECLINICAL</td>
</tr>
<tr>
<td>Palonosetron China</td>
<td>Chemotherapy-Induced Nausea and Vomiting</td>
<td>RESEARCH</td>
</tr>
<tr>
<td>Palonosetron</td>
<td>Chemotherapy-Induced Nausea and Vomiting in Pediatrics</td>
<td>RESEARCH PRECLINICAL</td>
</tr>
<tr>
<td>Anamorelin China</td>
<td>Non-Small Cell Lung Cancer associated cachexia/anorexia</td>
<td>RESEARCH PRECLINICAL</td>
</tr>
<tr>
<td>Anamorelin</td>
<td>Non-Small Cell Lung Cancer associated cachexia/anorexia</td>
<td>RESEARCH</td>
</tr>
<tr>
<td>Elsiglutide</td>
<td>Chemotherapy-Induced Diarrhea</td>
<td>REGULATORY APPROVED</td>
</tr>
<tr>
<td>Ghrelin Receptor Agonist 2</td>
<td>Cancer Cachexia/Anorexia</td>
<td></td>
</tr>
</tbody>
</table>

## Gastroenterology

<table>
<thead>
<tr>
<th>Drug</th>
<th>Condition</th>
<th>Status</th>
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</thead>
<tbody>
<tr>
<td>Peg 3350-Based OTC Laxative</td>
<td>Constipation</td>
<td>REGULATORY APPROVED</td>
</tr>
<tr>
<td>Ghrelin Receptor Agonist 1</td>
<td>Undisclosed</td>
<td></td>
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</table>

## Metabolism

<table>
<thead>
<tr>
<th>Drug</th>
<th>Condition</th>
<th>Status</th>
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</thead>
<tbody>
<tr>
<td>Ghrelin Receptor Antagonist</td>
<td>Obesity</td>
<td></td>
</tr>
<tr>
<td>Ghrelin Receptor Inverse Agonists</td>
<td>Obesity</td>
<td></td>
</tr>
</tbody>
</table>
Helsinn’s Product Development

- Integrated discovery/preclinical and clinical organizations
- R&D capabilities validated by continuous program successes and repeated licensing to partners
- Effective management of product development
- Two main phase III programs:
  - Anamorelin
  - Netupitant/Palonosetron
- 25% of revenue ($452 million) has been re-invested in R&D in the last five years
- 566 employees (313 in CH + 253 abroad) of which 21.2% are in R&D functions
Netupitant is a potent and selective NK1 receptor antagonist

Under development in combination with Palonosetron for the prevention of chemotherapy-induced nausea and vomiting (CINV)

Development status:
- Under registration at FDA and EMA
Under Development

CANCER ANOREXIA-CACHEXIA SYNDROME

Helsinn’s First Direct Sales Product; Launch is Targeted for 4Q 2015

- Anamorelin: being investigated for the treatment of anorexia/cachexia associated with non-small cell lung cancer (NSCLC)
- Development status: Phase III on-going
- Cancer anorexia-cachexia syndrome (CACS) is a multifactorial condition defined by an ongoing loss of body weight (mainly skeletal muscle mass, with or without loss of fat mass) that cannot be fully reversed by conventional nutritional support and leads to progressive functional impairment (1)

Incidence of cachexia in NSCLC patients is 50-66%

1) Fearon K et al, Lancet Oncol. 2011 May;12(5):489-95
Elsiglutide (ZP1846)

- A novel Glucagon-like Peptide-2 (GLP-2) analogue - 39 amino acid polypeptide
- Exerting trophic effects on the intestinal mucosa which is under development for the management of chemotherapy-induced diarrhea.

Development status: Phase IIa completed, entering Phase IIb
The Lead Optimization Program: the GHRELIN PLATFORM

**GHRELIN**: Ghrelin is a hormone recognized to have appetite-regulating properties

**GHRELIN RECEPTOR ANTAGONISTS**
One ghrelin receptor antagonist + several back-up compounds
**Development status**: Early pre-clinical (pre-IND) development

**GHRELIN RECEPTOR AGONISTS**
Two novel ghrelin agonists
**Development status**: Late pre-clinical (pre-IND) development

**IP Status**: Patented in China and Internationally
International Growth
Additions to portfolio include:

- Elsiglutide and compounds acting on the ghrelin receptor under development by Helsinn
- Additional compounds acquired through licensing and acquisition
Corporate Development & New Ventures in the USA

Other investments:
- Strategic participation in Thorne Research a US$ 60 mio sales company
- OncoQOL: enhancing Quality of Life through targeted Nutraceuticals
- HealthElements.com: a new web portal focused on Personalized Health Lifestyle, now WellnessFX

Participation in Windham Healthcare Fund:
- 100% life sciences, mostly medical devices but also Healthcare IT and late-stage selective therapeutics
Building a China Presence

PRESENCE IN CHINA FROM 2012

- Representative office in Beijing from end 2012
- Country manager on board with more than 10 years’ experience in China
- Development and regulatory activities for new generation products: IND for Netupitant-Palonosetron Fixed Dose Combination approved, Phase III started. Anamorelin IND submitted in September 2013
- Targeting direct sales in China from 2016-17

COORDINATION OF EXISTING ACTIVITIES

- Lead optimization R&D programs at Shanghai discovery labs
- Chemical supply chain management with a J-V partner
Manufacturing and Supply Chain
Helsinn Advanced Synthesis, Biasca, Switzerland

Integrated Manufacturing for Helsinn Group and Third Parties

- Full CMC development, industrialization and validation
- GMP intermediates
- API (Active Pharmaceuticals) Ingredients
- HPAI (High Potency Active Ingredients)
- Cytotoxic Compounds

- Approved by:
  - FDA
  - SwissMedic
  - EMA
  - KFDA (Korea)
  - PMDA (Japan)
6’000 sq. m facility in Damastown, technology park on the west side of Dublin, Ireland, and occupies a 27’000 sq. m site

2001 expansion to produce topical gel production

2003 commenced commercial secondary packaging of Palonosetron

2007 expanded by an additional 2’000 sq. m including a second vial packaging line and primary and secondary packaging of clinical materials (CTM)

2010 Opening of new OSD Centre and Stability Centre

Total site headcount approx. 180 (2014)

Approved by:
- EMA/IMB
- FDA
- EU-Pharmacovigilance

National Industrial Safety Award
Summary and Upcoming Milestones
2014 Projected Milestones

- Approval and launch of Netupitant-Palonosetron Fixed Dose Combination in US, PDUFA date 27 September
- Completion of Anamorelin phase III and preparation of regulatory submissions, data to be presented at ESMO in September
- Move our first second-generation Ghrelin agonist into phase I
- Target a range of IP and acquisition opportunities
- Finalize a new co-promotion agreement in US
- First year of co-detailing of Aloxi in US
- Complete worldwide licensing for Netupitant-Palonosetron Fixed Dose Combination and Anamorelin
- $360 million sales, with 23% reinvested into R&D and 600 employees
Helsinn in Summary

- A world-leading independent cancer company focused on cancer supportive care and quality of life products
- Private, well established profitable company with positive cash flow, no bank debt and a strong growth trajectory
- Broad portfolio of established products with CINV market leader, Aloxi
- Unique R&D pipeline of technologies and new products in cancer care
- Building out into new international territories and investing in innovative technologies
- Family run firm with focused management team

A GLOBAL LEADING COMPANY IN CANCER SUPPORTIVE CARE
Building quality cancer care together

Thank you