

(NYSE Euronext: PHARM)

Sijmen de Vries, MD, MBA Chief Executive Officer

Annual General Meeting of Shareholders Leiden, 18 June 2014.

**PHARMING** 



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# **Operations 2013**

- Certification by EMA for 2<sup>nd</sup> downstream production facility at Sanofi- Chimie
  - Up- scaling of production capacity and reduction in cost of goods
  - Manufacturing of inventories for US launch was initiated 2H2013
- Restructuring of organisation completed, in addition significant savings achieved from reduction of infrastructure
  - Significant downsizing of Dutch operations and facilities
  - Total headcount YE2013; 44 (YE 2012:61)
- Ruconest BLA for acute HAE submitted and accepted for review by FDA
  - US\$5M milestone payment from SNTS was received
- Entered into strategic product development collaboration with Shanghai Institute for Pharmaceutical Industry (SIPI)
  - Future new product development using Pharming platform at SIPI
  - Duplication of C1INH manufacturing operations; future 2<sup>nd</sup> source for Ruconest
  - Development of rhFVIII for Haemophilia A

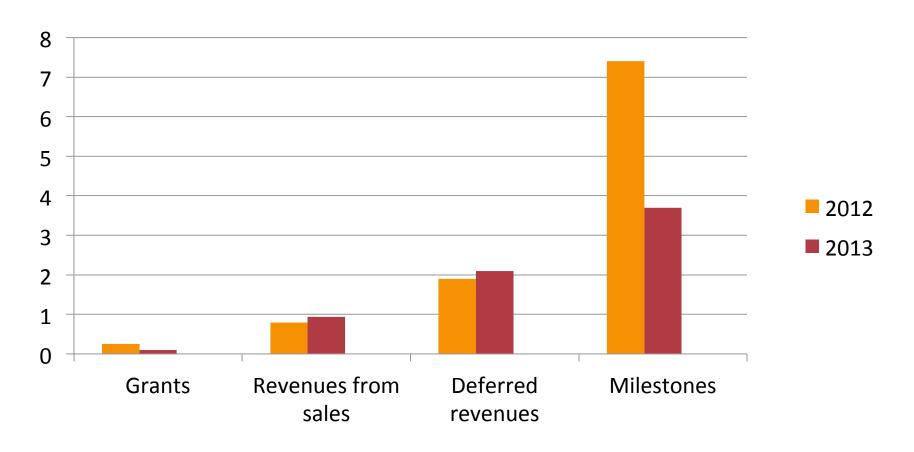
## **Operations 2013**

- EU rollout continuing by partner Sobi
  - Improving in- market sales under challenging EU market access conditions
    - FY2013 revenues from sales to Sobi at €0.9M
  - Significant market penetration in several Central/ Eastern European markets
  - Consistent and significant repeat use and high patient and physician satisfaction
- Approved in Israel/ final stage of regulatory review in Turkey
  - Partnered with Megapharm (Israel) and Eczasibasi (Turkey)
  - SE- Asian territories partnered with Transmedic Pte. and Hyupjin Corp.
  - China, Taiwan, Hong Kong and Macau partnered with SIPI
- Unlimited supply capabilities and significant economies of scale
  - Rapidly scale- able supply chain
  - Technology transfer to SIPI to set- up future second supply source in Shanghai

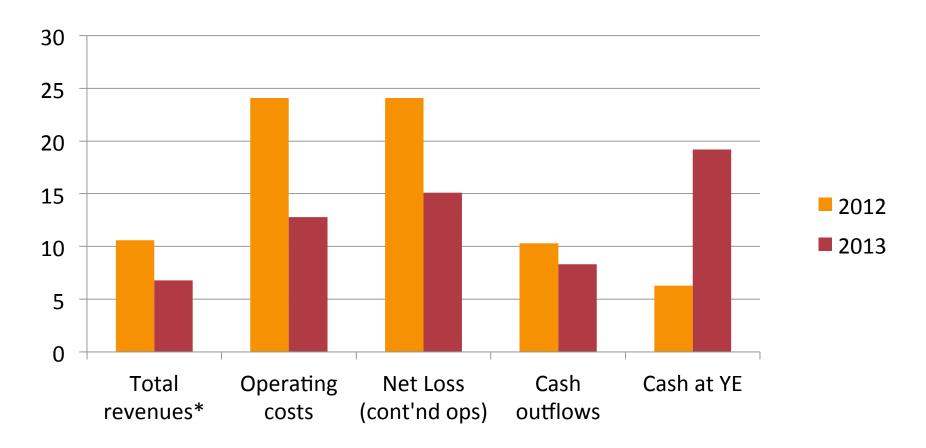
# Corporate Social responsibility/ sustainability

- Medical need and patient safety
- Code of Conduct
- Animal care code of conduct and animal welfare
- Environmental impact of operations and trace- ability of supply chain
- Diversity and equal opportunities

## Financial headlines: Revenues 2013



### **Financial headlines 2013**



<sup>\*</sup>Including SNTS milestone payments (2012 \$10 million/ 2013 \$5 million)

# **Dutch Corporate Governance Code**

Corporate Governance Statement 2014 on website <a href="www.pharming.com/corporate">www.pharming.com/corporate</a>

Pharming complies with the Dutch Corporate Governance Code except for sections:

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II.2.4. (Options for the Management board)
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II.2.6. (Option exercise price)

III.3.1. (Profile of the Board of Supervisory Directors)

**III.5.14** (Selection and appointment committee)

III.6.5 (Ownership and transactions in securities other than issued by the Company)

III.7.1. (Shares for the Supervisory Board of Directors)

IV.3.1 (Follow in real-time all the meetings)

IV.3.12 (Independent third party to hold proxies)

IV.3.13 (Outline policy in bilateral contact with shareholders)

III.5.4c-III.5.4d and V.3.1.-V3.3. (Internal auditor)

# Risk management and controls

#### Periodical risk assessments and reviews

- Types of risk assessed (in no particular order)
- Macro (economical), Clinical and Regulatory, Research, Manufacturing,
   Commercial, Financial, IT, Human Resources

#### Financial control systems

- All revenues are generated and controlled by mother company
- Expenses and capital expenditures regulated by chart of authority

### **Business model**

- Future profitability initially driven by
- Proceeds from Ruconest US sales
  - Tiered supply price to SLXP: 30- 40% of net sales
- Proceeds from Ruconest EU sales
  - Fixed supply price per vial to Sobi
- Potential for increases of profitability/ vial as results of
  - Economies of scale in current manufacturing process (Sanofi)
  - Future supplies from 2<sup>nd</sup> manufacturing site at SIPI
- Potential for increasing profitability from development of Ruconest in additional indications (eg. Prophylaxis of HAE, Acute Pancreatitis, DGF)
- Competition
  - Intense and embedded competition, continuous innovation
  - Long development cycles and high hurdles for entry (no "surprise entries")
  - Risk of rapid erosion of profitability as result of new entries

## US market: Rapid growth, significant potential

- HAE disease awareness in the US continues to improve, leading to more patient identification\*
- FY 2013 sales for acute treatment increased to approx. US\$ 275M from US\$ 156M for FY2012 (50% growth) excluding Berinert® sales (not disclosed\*\*\*)
  - US\$ 235M Firazyr® (US\$ 116M; 2012) and 1Q2014 sales of \$75M\*\*
  - US\$ 40.5M Kalbitor® (US\$ 39.8M;2012) and 1Q2014 sales of \$12.5M\*\*
  - Treatment costs estimated at US\$70k/ annum\*\*\*
- FY 2013 sales for prophylaxis (Cinryze®) increased to approx. US\$395M from US\$327M for FY 2012 and 2M 2014 sales of \$86M \*\*
- More patients seeking treatment for moderate symptoms\*
  - Guidelines recommend treating all attacks since any one could become severe
  - Many patients use multiple products, patient driven therapies
  - Significant steroid usage remains to date

<sup>\*</sup> Leerink Swann, competitor interviews, 13 Sept13, 2012,

<sup>\*\*</sup> Quarterly results 2013/ 2014, analyst estimates and FY 2013 results 10-Q filings DYAX, SHPG

<sup>\*\*\*</sup> Seeking alpha an overview of HAE 18 Sep 2012

### **HAE** treatment options (published data)

		recombinant C1 Inhibitor	plasma derived C1 Inhibitor		bradykinin receptor antagonist	kallikrein inhibitor
		Ruconest <sup>^</sup>	Cinryze^^	Berinert	Firazyr**	Kalbitor^^^
Efficacy		Excellent	Good	Good	Good	Good
	Dosing (C1INH)	50 U/kg*	~ 12 U/kg	20 U/kg		
	Treatment type	Any acute	Prophylaxis	Limited****	Any acute	Any acute
	Response < 4h	80-100%	~ 60%	70%	58-74%	73%
Safety concerns		Very low risk of allergic reaction	Warning: Risk of blood clots	Warning: Risk of blood clots	97% injection site reactions	Black box warning 3.9% anaphylaxis
	Plasma risk	NO	YES	YES	No	No
Purity (C1INH)		>99.9%	±80%	±95%		
Relapse / worsening		Uncommon	Uncommon	Uncommon	11-31%***	21%
Administration		IV	IV	IV	SQ	SQ (no self-administration)

<sup>\*</sup>Optimal efficacy of C1INH therapy is achieved at doses ≥50 U/kg ("Target levels of functional C1-inhibitor in hereditary Angioedema". Allergy, C. E. Hack, A. Relan, E. S. van Amersfoort & M. Cicardi)

\*\*\*\*Berinert not licensed for peripheral attacks in the US,

<sup>\*\*</sup>Icatibant Clinical Briefing Document, CDER, FDA, 2011./ Aberer, et al. Ann Allergy Asthma Immunol 2010; 105(5):P238

<sup>\*\* \*</sup>Cicardi et al, N Engl J Med 2010;363:532-41.; Aberer, et al. Ann Allergy Asthma Immunol 2010; 105(5):P238; Lumry, et al. Ann Allergy Asthma Immunol. 2011;107:529 –537.

<sup>^</sup>Ruconest approved in EU and Israel, ^^Cinryze not licensed for acute therapy in US. ^^^Kalbitor not approved in EU.

# **Technology Platform**

# Strategic product development collaboration

SIPI (Shanghai Institute for Pharmaceutical Industry: A Sinopharm company)

- Product development at SIPI
  - Under Pharming's fully ICH compliant QA systems
  - Compliant with CFDA, FDA and EMA standards
  - Funded by SIPI up to IND
  - Aligned clinical development (SIPI funds China/ Pharming funds ROW)
- Technology transfer of Pharming platform to SIPI facilities in Shanghai
  - Initial projects C-1 Inhibitor and Factor VIII
  - Includes manufacturing of (future) finished products
- SIPI's product development resources and SIPI's favourable cost structures for development and manufacturing combined with the competitive features of the platform

### **SIPI** collaboration

- Commercialisation rights: SIPI China/ Pharming ROW
  - Reciprocal royalties at 4%: SIPI (China)/ Pharming (ROW)
  - SIPI to pay product related milestones for all future products developed
  - SIPI to supply Pharming on "cost plus" basis for ROW
- SIPI pays €1.26 million upfront and € 0.84 million technology transfer fees and all Pharming technology transfer related expenses
- SIPI pays €0.3 million at receipt of Ruconest drug importation license
  - Until completion of technology transfer, Pharming to supply SIPI with Ruconest as imported product ("cost plus" basis and 4% royalties)

# **Outlook 2014 and beyond**

- PDUFA date Ruconest® for acute HAE 16 July 2014.
  - Differentiated competitive profile/ Rapidly expanding US acute market segment estimated at
     >US\$ 400M + per annum
  - Significant potential near term milestone US\$ 20M (first US commercial sale)
  - Revenues from US net sales between 30-40%
- Significant up- side potential from additional indications
  - Prophylaxis of HAE and Acute Pancreatitis
- Ruconest® sales increasing in Europe and ROW
  - Ex- US revenues (2014) from sales expected at €3M
- Pipeline development
  - New product development at SIPI and supply by SIPI
- Stabilised balance sheet + low operating costs:
   Basis for future profitability
  - Increasing ROW sales and US market entry to drive economies of scale/reduction of COGS
  - Significant value inflexion points ahead

# www.pharming.com

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