# Pharming Group NV

Extraordinary General Meeting of Shareholders

Sijmen de Vries, CEO
Bruno Giannetti, COO
Robin Wright, CFO

Leiden
Netherlands
05 October 2016



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## On 9 August, Pharming announced that it will be buying back the North American commercial rights to RUCONEST® from Valeant

- Deal value is $125 million, with an upfront of $60 million
- Self funding Milestones on sales up to a maximum of $65 million
- Deal is subject to financing
- Strong interest from investors wanting to back equity or debt
- Main reason for deal is to enable us to focus on realizing RUCONEST® potential, leveraging our 10+ years experience of US HAE networks
- Expect to close on the transaction in November 2016



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# US HAE Market Features



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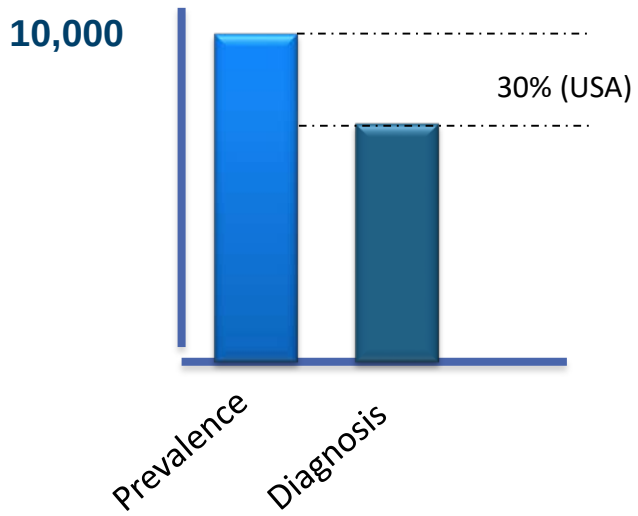
# Hereditary Angioedema (HAE)

- Rare genetic disorder caused by mutations in the gene encoding C1-esterase inhibitor (C1INH)
  - Patients present with swelling, severe abdominal pain, or acute airway obstruction
  - Can be fatal if left untreated




- Prevalence estimates range from 1 in 10K–50K
- 75% of patients present before age 15
- Misdiagnosis is common; rate of diagnosis varies widely (US is most developed market)
- Growth comes from better prescription and better diagnosis rates
- Increase in attacks treated per patient per year
  - Still significant long term steroid prophylaxis
    - Ineffective, with high liver toxicity risk
  - Laryngeal attacks are potentially fatal
  - More aggressive treatment of all type of attacks

Inhibition of C1 esterase is Gold Standard for HAE treatment (protein replacement)



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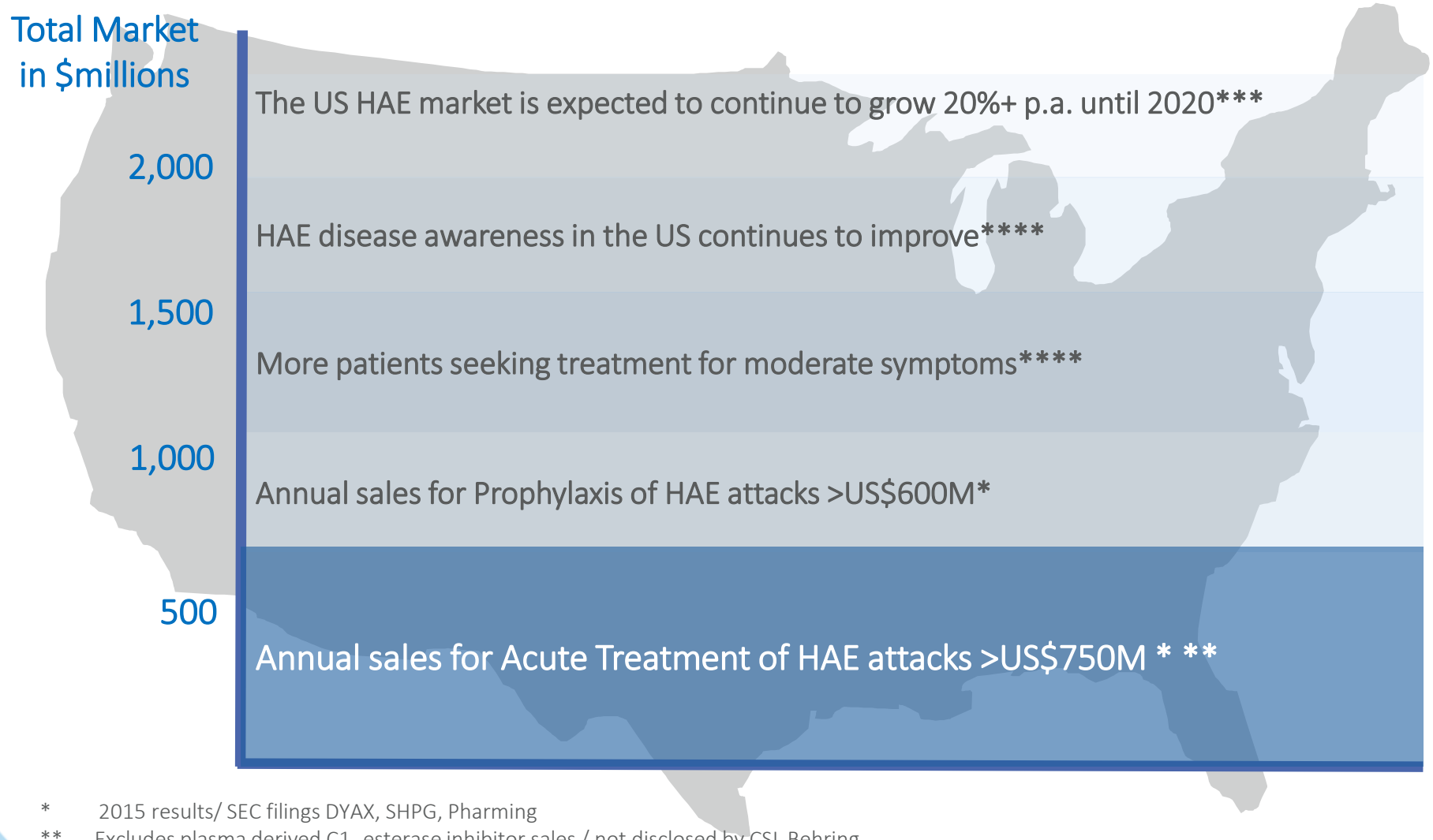
# US HAE market: Rapid Growth, Significant Potential




* 2015 results/ SEC filings DYAX, SHPG, Pharming
** Excludes plasma derived C1- esterase inhibitor sales / not disclosed by CSL Behring
*** Valeant competitor analysis, September 2015
**** Leerink Swann, competitor interviews, 13 September 2012



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# HAE Treatment Options (Published Data except sales)

| | | Recombinant C1 Inhibitor | Plasma-derived C1 Inhibitor concentrates | | Bradykinin receptor antagonist | Kallikrein inhibitor | Clinical Trial: Kallikrein inhibitor antibody |
|---|---|---|---|---|---|---|---|
| Names | | RUCONEST® ^ | Cinryze^^^ | Berinert | Firazyr** | Kalbitor^^^^ | DX 2930 (a.k.a. SHP643) |
| Owner | | Pharming | Shire | CSL Behring | Shire | Shire | Shire |
| Sales† | | $33m | $550m | $200m | $500m | $83m | Entering Phase III |
| Efficacy | | Good & consistent | Good | Good | Good | Good | |
| | Dosing (C1INH) | 50 U/kg* | ~ 12 U/kg | 20 U/kg | N/A | N/A | N/A |
| | Treatment type | Acute^^ | Prophylaxis | Acute**** | Acute | Acute | Prophylaxis |
| | Response < 4h | 89% | ~ 52% | 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 | Data is in mild patients only |
| | Plasma risk | NO | YES | YES | N/A | N/A | N/A |
| Purity (C1INH) | | >99.9% | ±80% | ±95% | | | |
| Relapse / worsening | | Uncommon | Uncommon | Uncommon | 11-31%*** | 17% | ?? |
| Administration | | IV (SC, IM coming) | Twice weekly IV | IV (SC coming) | SC | SC (Hospital only) | SC |

† Sales figures are Pharming estimates based on relevant selling company's releases and financial reports as well as IMS data and other proprietary databases

*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)

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

** *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.

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

^Ruconest approved in US, EU and Israel, ^^Ruconest filed for laryngeal attacks (US), ^^^Cinryze not licensed for acute therapy in US. ^^^^Kalbitor not approved in EU.

?? Kalbitor moderate response rate is likely to be pathway-related, at least in part. Relapse rate is also likely to be pathway-related in part. Accordingly DX 2930 may also have these issues. In addition, the safety consequences of chronically inhibiting the contact pathway have not been studied, and this may also be a factor. Antibodies tend not to have large (>75%) response rates.

Note: New forms of products for different routes of administration may require clinical development and regulatory approval.



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# Next indication in development: Prophylaxis of HAE



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# RUCONEST® - Prophylaxis of HAE

- Phase II results meet primary endpoints for once- and twice weekly regimen and show that twice-weekly prophylaxis treatment significantly (-72%) reduces attack frequency and features a 96% response rate (>50% reduction of attack frequency)
- The only approved product, a blood derived C1- inhibitor concentrate dosed twice weekly reduces attacks by 52% and has a 50% response rate*
- RUCONEST® also approved for acute attacks, and so can become its own rescue therapy

| | | Placebo | RUCONEST® | RUCONEST® |
|---|---|---|---|---|
| Intent-to-Treat Analysis | | | Once/week | Twice/week |
| (n=32) | Primary: Mean number of attacks | 7.2 | 4.4 | 2.7 |
| | Confidence Interval (95%) | 5.8-8.6 | 3.1-5.6 | 1.8-3.7 |
| | *p-value* | | *0.0004* | *p<0.0001* |
| (n=31) | Secondary: % Patients with more than 50% reduction in attack frequency | | 42% | 74% |
| | Confidence Interval (95%) | | 26-59 | 57-86 |
| Per Protocol Analysis | | | | |
| (n=23) | Mean number of attacks | 7.5 | 3.8 | 2 |
| | Confidence Interval (95%) | 6.0-9.0 | 2.5-5.1 | 1.3-2.7 |
| | *p-value* | | *p<0.0001* | *p<0.0001* |
| (n=23) | % Patients with more than 50% reduction in attack frequency | | 57% | 96% |
| | Confidence Interval (95%) | | 37-74 | 79-99 |

*Zuraw et al; Nanofiltered C1- inhibitor concentrate for the treatment of HAE: NEJM 363;6 (August 2010): pp 513-522



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# Transaction Overview



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# Commercial Plan Highlights I

**Market**

- US Market c. $1.4 billion, growth 20%-30% p.a., 50:50 Acute/Prophylaxis
- 4 acute products, Firazyr, Berinert, Kalbitor, Ruconest
- 1 prophylaxis (Cinryze). Many patients still on older therapies, e.g. Danazol
- Subcutaneous (or non-IV) is method of administration preferred by patients
- Positive Phase II prophylaxis data on RUCONEST reported (July 2016)

**Sales**

- US$24 million net sales in 2015 - mostly to early adopters so far
- Valeant reduced sales force from 24 reps in May 2015 to 8 reps in Oct 2015.
- Annualized sales run rate on basis of July- August 2016: US$33 million



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# Commercial Plan Highlights II

**Operations and Governance**

- Acquiring essentially the entire Valeant team as part of transaction
- Transition Services Agreement is place to ensure seamless transition
- Will use a Commercial Advisory Board to determine/monitor strategy in US
- Will open small US office in New Jersey

**Team**

- Expect to start with 11 Valeant sales staff
- Nursing hub: Independent nursing support and benefit verification services
- RUCONEST SOLUTIONS total care plan for patients



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# RUCONEST®

**Focus on the areas where RUCONEST® can differentiate:**

- Response rate – consistent and reliable: 90% of attacks treated first time with one dose
- Potential for development of Prophylaxis (once/ twice weekly)
- Fast time to onset of relief
- Favourable safety profile
- Viral safety (no risks of blood borne pathogens)
- Patient start-up and co-payment support (RUCONEST SOLUTIONS)
- New convenient versions on the horizon (IV Lite, IM, subcutaneous)



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# Building on the existing Valeant Sales Team

- The Valeant team available to Pharming comprises of 11 sales staff in total
- VP of US operations to be added
- Additional sales staff/ sales management to be added
- MSL team to be added
- Marketing and professional relations functions
- Reimbursement/ payer management function
- Patient access managers and patient advocates
- Extension of specialty pharmacy distribution network



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# US management structure

- New **Commercial Advisory Board** will set strategy, monitor performance and provide US Commercial experience
- Initial members:
  - Experienced ex-CEO of US public company
  - Sijmen de Vries, CEO, Pharming
  - VP Pharming US operations
  - Experienced US HAE sales & marketing expert(s)
- VP Pharming US operations will be responsible for day-to-day management of team
- Finance, HR, IT, Communications, Legal and Regulatory will be run out of Leiden at first, with outsourcing wherever beneficial to suppliers in the USA
- New office in New Jersey for effective cost/communications



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# Financial Summary – 8 months to 31 August 2016

| Amounts in €m, except per share data | First 8 months 2016 | First 8 months 2015 | % Change | H1 2016 | % Change |
|---|---|---|---|---|---|
| *Income Statement* | | | | | |
| Product sales | 6.2 | 5.6 | *11%* | 4.2 | *48%* |
| License fees | 1.5 | 1.5 | - | 1.1 | *36%* |
| Revenue | 7.7 | 7.1 | *8%* | 5.3 | *45%* |
| Gross Profit | 4.7 | 3.8 | *24%* | 3.3 | *42%* |
| Costs | (13.2) | (12.0) | *(10%)* | (9.7) | *(36%)* |
| Operating Result | (8.3) | (8.1) | *(3%)* | (6.2) | *(34%)* |
| *Balance Sheet* | | | | | |
| Cash & marketable securities | 18.1 | 35.4 | *(49%)* | 21.7 | *(17%)* |
| *Share Information* | | | | | |
| Earnings per share | (0.022) | (0.014) | *(36%)* | (0.016) | *(38%)* |

*Note: €1 = $1.12*



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# Pro forma at Half-Year and 8 Months 2016*

| Amounts in €m (unaudited) except per share data | Actual YTD 2016 | *Pro Forma* YTD 2016 | % Net Change | *Pro Forma* 1H 2016 | % Net Change* |
|---|---|---|---|---|---|
| *Income Statement* | | | | | |
| Product sales | 6.2 | 17.7 | *186%* | 12.4 | *195%* |
| License fees | 1.5 | 0.7 | *(53%)* | 1.1 | - |
| Revenue | 7.7 | 18.4 | *139%* | 13.5 | *155%* |
| Gross Profit | 4.7 | 15.4 | *228%* | 11.5 | *248%* |
| Costs | (13.0) | (18.6) | *(43)%* | (14.7) | *52%* |
| Operating Result | (8.3) | (3.2) | *61%* | (3.1) | *50%* |
| *Balance Sheet* | | | | | |
| Cash & marketable securities | 18.1 | 23.6 | *30%* | 26.3 | *21%* |
| *Share Information* | | | | | |
| Earnings per share | (0.022) | (0.013) | *42%* | (0.013) | *19%* |

* For comparison and illustrative purposes only; on the basis that the Valeant transaction had been completed as at 01 January 2016



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# Structuring



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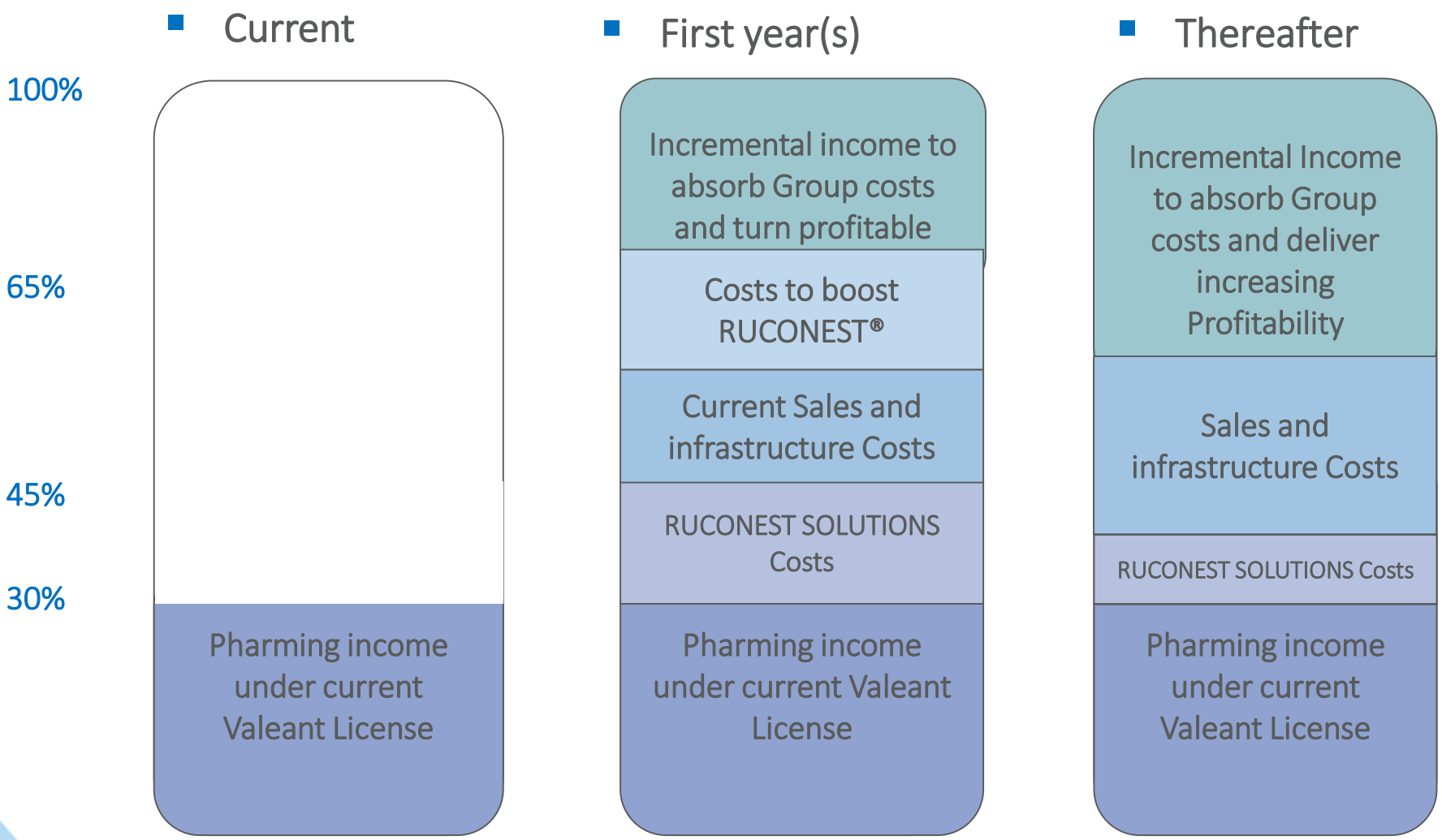
# Financial Structuring:



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# Financial Structuring:

## Need

### NOW

- Upfront for Valeant of US$60 million
- Working capital to invest in RUCONEST® to improve sales in USA and ROW  US$14-20 million
- Additional working capital up to US$12 million
- Transaction costs US$6-8 million

**TOTAL** **US$80-100 million**

## Sources

### NOW

- DEBT: New straight debt facility expected c. US$30-40 million (or ~US$50 million if current Oxford Finance & Silicon Valley Bank facility replaced)
- EQUITY-1: Convertible Bond issue for institutional investors
- EQUITY-2: Rights Issue for existing shareholders and underwritten by institutional investors

**TOTAL** **US$80-100 million**



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# Current thinking on Equity part of transaction:

- Rights Issue
  - Relatively small rights discount of around 10-15%
  - All shares will be offered to existing shareholders in a rights issue
  - Rights will be tradable
- Convertible Bond:
  - Conversion priced at a premium to share price
  - c.6% coupon, callable and redeemable at a premium, otherwise normal
  - Bonds could be tradable
- Supporting investors would only be eligible once shareholders have taken up all the rights they wish, but accepted convertible orders would be binding subject to Closing
- Allocation of Equity between Rights Issue and Convertible Bond may fluctuate depending on market conditions and as we seek to minimize the amount of dilution for shareholders



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# What's in it for Pharming Shareholders?



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# Attractive Growth Proposition

- We believe RUCONEST® can be sold better by Pharming, as we are able to focus and are able to put the needed specific resources behind the sales team
- Pharming has an excellent reputation in the HAE space, and strong support from the patients‘ associations
- This transaction will make Pharming profitable at operating level in 2017 even if sales only stay close to July- August 2016 levels
- The timing of reaching profitability and the level of profitability depends on sales patterns during the rest of 2016 and 2017
- With focused investment and expansion of MSLs and sales reps, we believe we can get very significant sales growth and expect to be able to generate very substantial future Earnings
- Our next (rare disease) products are expected to come online in 2020-2021 after development, so we would have a US commercial operation capable of selling those immediately ourselves



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