Pharming Group NV

Robin Wright
Chief Financial Officer

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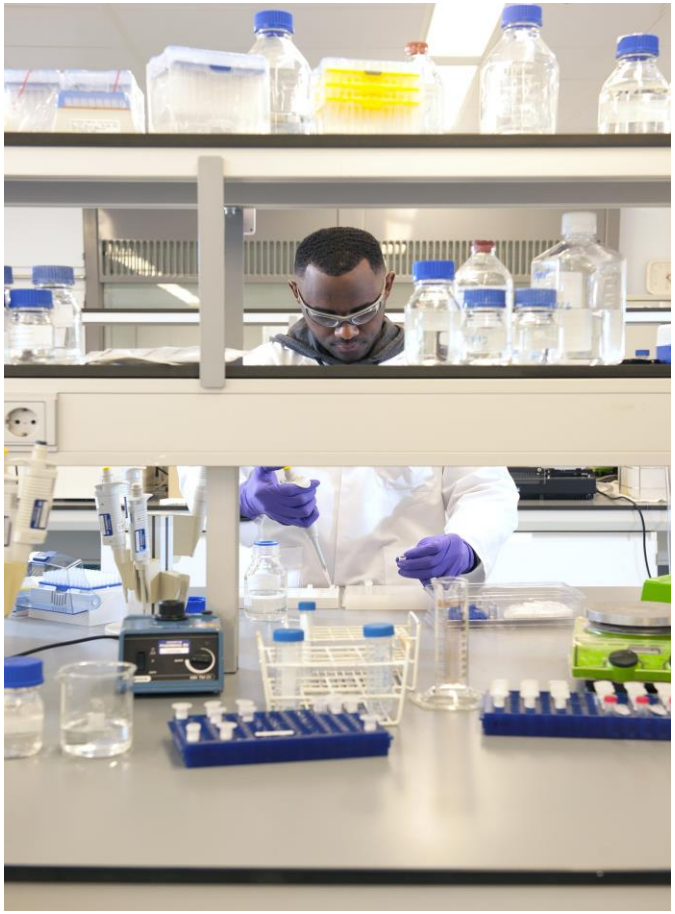
Company Overview

- Euronext: PHARM: ~€500 million (~$564 million) at €0.80 per share
- Headquarters in NL, R&D in France, EU and US: 200 employees in total
- 1st product approved and marketed : RUCONEST®
  - Recombinant human C1-esterase inhibitor (enzyme replacement therapy)
  - For acute angioedema attacks in patients with hereditary angioedema (HAE)
  - Marketed in USA, EU, LatAm, Korea and Israel with other territories coming
  - Imminently entering clinical development for additional large unmet indications
- Platform technology makes recombinant human molecules cleanly and efficiently
- New Enzyme Replacement Therapies (ERT) for other genetic conditions (Pompe, Fabry) to start entering clinic once file and manufacturing complete



Growth continues for RUCONEST® in changing HAE landscape

- Recent launches of subcutaneous injected plasma-derived C1 inhibitor product and subcutaneous long-acting antibody increase future competitive pressure in the prophylaxis segment
- New treatments feature higher responder rates than previous IV plasma-derived C1-inhibitor prophylaxis
- Continued need for effective and reliable treatment for breakthrough attacks, because over half of patients continue to have breakthrough attacks
- The management of HAE typically improves by having multiple types of treatments at hand over time
- Reliable and consistent response when treating attacks of HAE, and the corresponding increasing positive patient experiences, means that RUCONEST continues to find its place as preferred/ ultimate treatment
- Opportunity now both for severely-affected patients and for treatment of breakthrough attacks associated with the new prophylaxis products, providing scope for continued growth

Strengthening RUCONEST's position in acute HAE

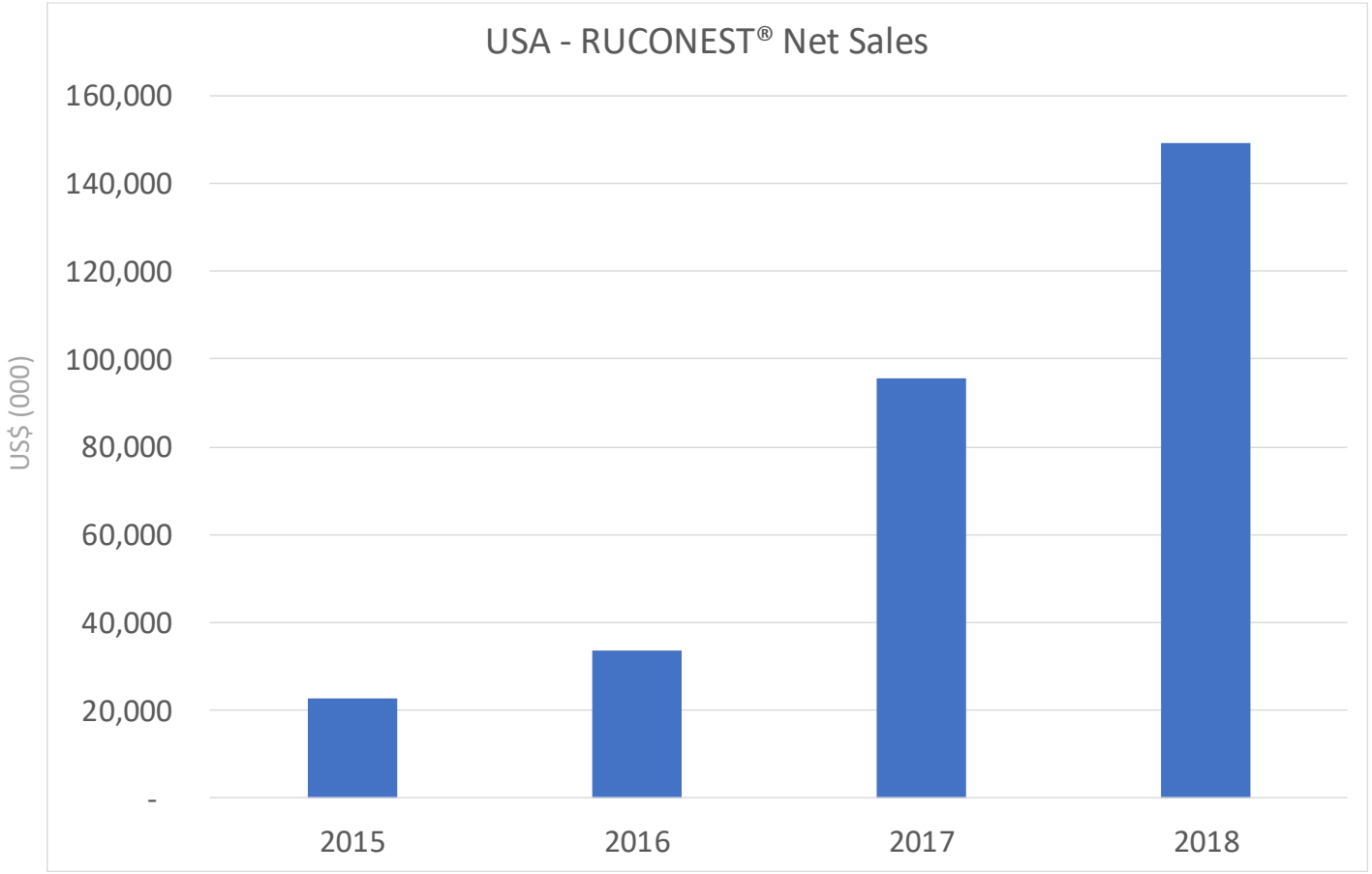
- Publications at various scientific congresses throughout the year further underpinning reliability and consistency of response to RUCONEST therapy
- These include the first investigator-initiated (observational) real-world study comparing re-dosing frequency of C1 esterase inhibitor therapies versus icatibant (bradykinin inhibition) therapy in 69 acute HAE attacks
- The data showed that properly dosed, RUCONEST® and other C1 esterase therapies would typically stop HAE attacks on the first treatment and that icatibant (marketed as the world's best-selling HAE drug, Firazyr®) required frequent (and multiple) re-dosing to treat an attack of HAE
- The full results are being written up now by the investigators now for publication shortly

Full Year 2018: Preliminary Financial Results

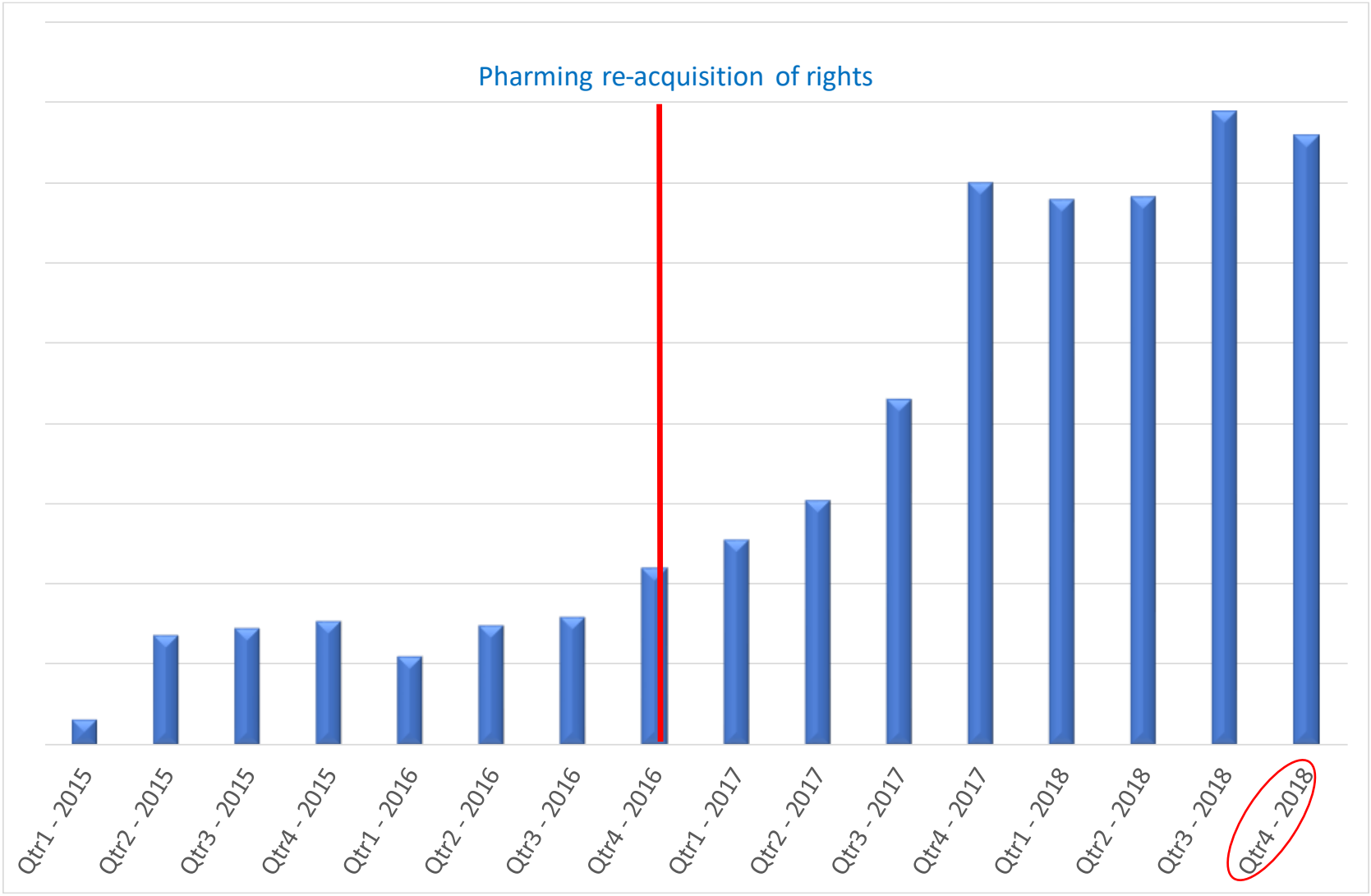
Amounts in €m except per share data	*2018*	*2017 Restated***	*% Change*
Income Statement			
Product Sales	134.3	88.7	*51%*
License Revenue	0.8	0.9	*(15%)*
Total Revenue	135.1	89.6	*51%*
Gross profit	113.0	77.2	*46%*
Operating result	38.0	21.9	*74%*
Financial Income, expenses and adjustments	(37.1)	(107.6)	*(66%)*
Tax credit/(expense)	24.1	9.4	*n/a**
Net result	25.0	(76.2)	
Balance Sheet			
Cash & marketable securities	81.5	60.0	*36%*
Share Information			
Earnings per share before dilution (€)	0.041	(0.152)	*126%*

Strong execution of commercial strategy

- Revenues from product sales for the year increased by 51.2% to €134.1 million (FY 2017: €88.7 million), as a result of increasing patient numbers
- The effect of Pharming's approach to US commercialisation can be seen in the chart below:



US quarterly sales development in volumes

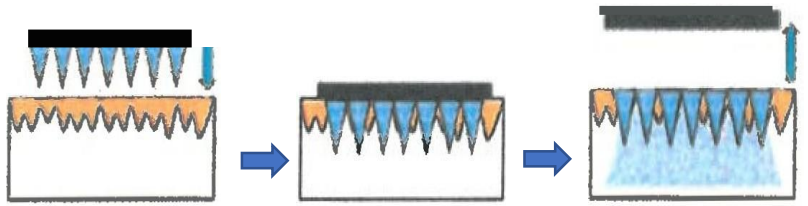


Three pillars for strong organic growth

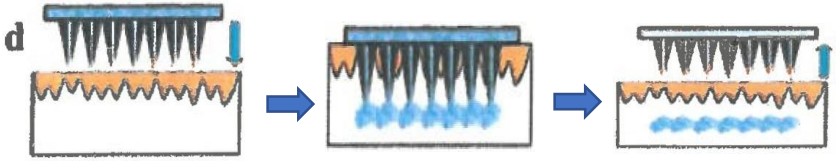
- Focusing on investment in our three pillars of organic growth outlined at the Capital Markets Day in June 2018:
  - Convenience of RUCONEST® within the HAE space to meet patients' needs – new intramuscular, subcutaneous and intradermal versions under development
  - Development of RUCONEST®/rhC1INH outside the HAE space to tackle large unmet medical needs for which there are no current approved or effective therapies: initially pre-eclampsia and acute kidney injury
  - New protein replacement products which address significant shortcomings of existing therapies for Pompe and Fabry diseases
- Received a complete response letter for the use of RUCONEST® for the prophylaxis of HAE in September 2018, as result of the FDA being unable to cross the final remaining statistical hurdle in a small sub-group of patients.
  - This issue will be addressed as part of new prophylaxis studies with more convenient forms of RUCONEST as outlined above

Formulation development of RUCONEST®

- The "RUCONEST® liquid" formulation (14ml → 3ml) can be used as starting material for the generation of subcutaneous, intra-muscular & intradermal application systems
- New proprietary 'painless' intradermal delivery applications are being developed:
- Dissolving point device:



- Reservoir device:



- These painless versions should differentiate RUCONEST® from competitors, all of which involve painful injections

New indications for RUCONEST® - Acute Kidney Injury

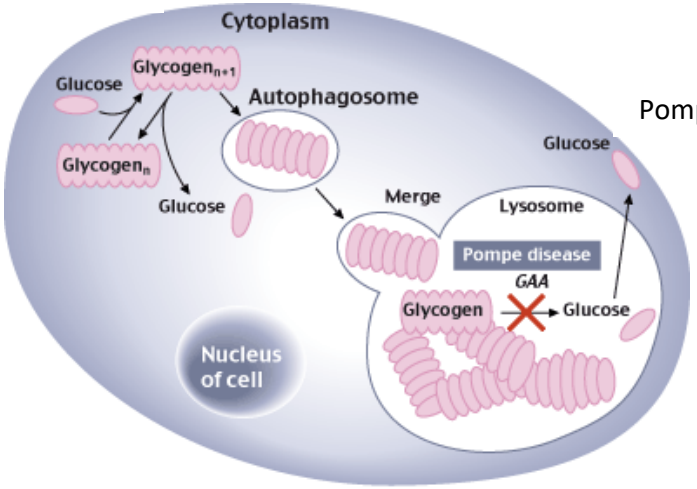
- Positive results delivered in October from a Phase II investigator-initiated study of RUCONEST® in a double-blind, placebo-controlled clinical trial in patients at risk of nephropathy resulting from contrast-enhanced examinations
- The result show clear clinically-significant benefit in patients undergoing percutaneous coronary interventions (“PCI”) such as stent insertions or valve replacements
- The intent-to-treat analysis showed that patients on RUCONEST® had a median percentage change in peak urinary Neutrophil Gelatinase-Associated Lipocalin within 48 hours of 11.3% in the RUCONEST® arm and 205.2% in the placebo arm (p=0.001)
- The overall assessment of the study also showed trends that patients undergoing more invasive interventions and procedures requiring higher volumes of contrast medium experienced a stronger benefit from the RUCONEST® treatment
- As a result, Pharming is now embarking on its own program for acute kidney injury in patients undergoing PCIs accompanied by contrast-enhanced examinations

New indications for RUCONEST® - Pre-eclampsia

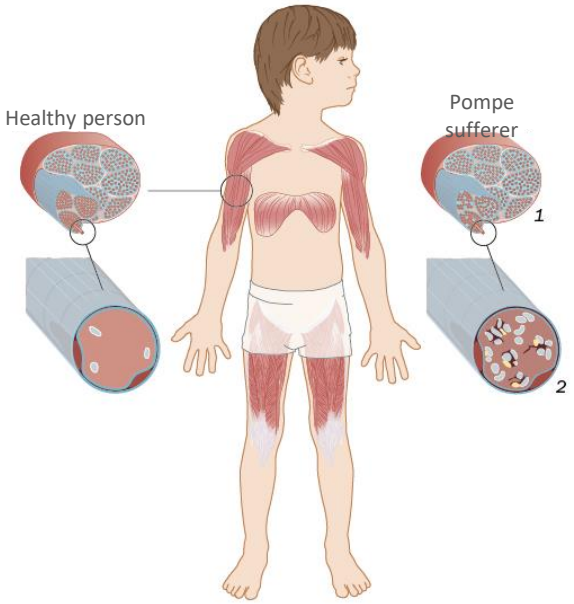
- An initial study to investigate safety (and efficacy signals) in pregnant women diagnosed with pre-eclampsia has been designed and was submitted to the regulatory authorities and ethics committees in Netherlands and Australia last year
- All study preparations are completed
- Any study in pregnant women, and especially studies in distressed pregnant women, are necessarily very carefully designed, planned and reviewed
- Now awaiting confirmation for final approvals to initiate the study from the relevant ethics committees
- Following ethics committee approval, the study is expected to start soon
- Ethics committee approval and first patient in will be reported by press release

Pompe disease

Normal situation



Pompe disease



- Progressive decrease in muscle strength starting with the legs and moving to smaller muscles in the trunk and arms, such as the diaphragm and other muscles required for breathing
- Cardiac failure and respiratory failure are the most common causes of death

Pompe disease

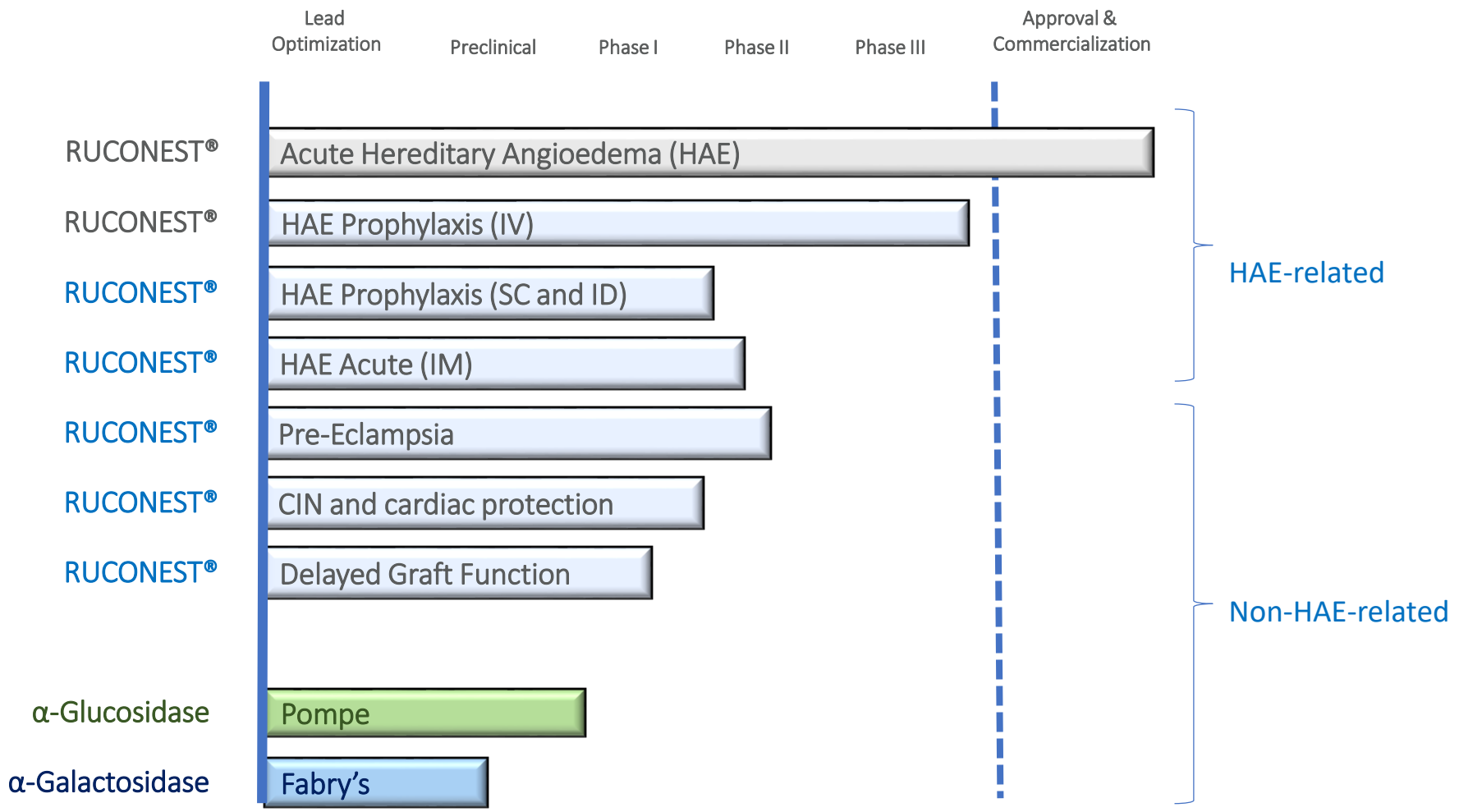
α-glucosidase

Market potential for Pharming is over $2 billion per year in each indication

- Attractive market:
  - Many patients (60-80%) are not on therapy because of antibody formation or adverse reactions
  - All current products have severe shortcomings and boxed warnings, but together sell for over $1 billion - 2nd generation products have their own shortcomings
  - rhC1INH RUCONEST (equally highly-glycosylated) from the same transgenic platform does not generate relevant antibody responses
  - A small clinical trial in infants with previous transgenic rhαGLU showed good efficacy with no reported safety concerns (2001)*
- GMP manufacturing and upscaling production to produce material for IND enabling studies and clinical trials
- KOL input and FDA/ EMA feed-back on clinical development design will determine start of clinical development
  - Phase I or direct to Phase I/II multiple dose (duration of IND enabling studies and upscaled manufacturing for Phase I/II multiple dose)

*Van den Hout et al., J. Inherit. Metab. Dis., 24 (2001), 266-274

Expansion of pipeline to multiple products/markets



Outlook for 2019

- Continued growth in revenues from sales of RUCONEST®, mainly driven by the US and Western Europe operations
- Continued achievement of positive net earnings during the year
- Continued investment in the expansion of production of RUCONEST® in order to meet the growing demand for RUCONEST® internationally
- Investment in further clinical trial programs for RUCONEST® with low-volume liquid intramuscular and subcutaneous versions of RUCONEST® for both acute treatment and prophylaxis of HAE, as well as research into other more convenient routes of administration.
- Investment in clinical trials for additional indications for RUCONEST®
- Investment in development of the new pipeline programs in Pompe disease and Fabry's disease, and other new development opportunities and assets as these occur
- Increasing marketing activity where this can be profitable for Pharming, such as opening new countries for RUCONEST®

No further financial guidance for 2019 is provided.

www.pharming.com

r.wright@pharming.com

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