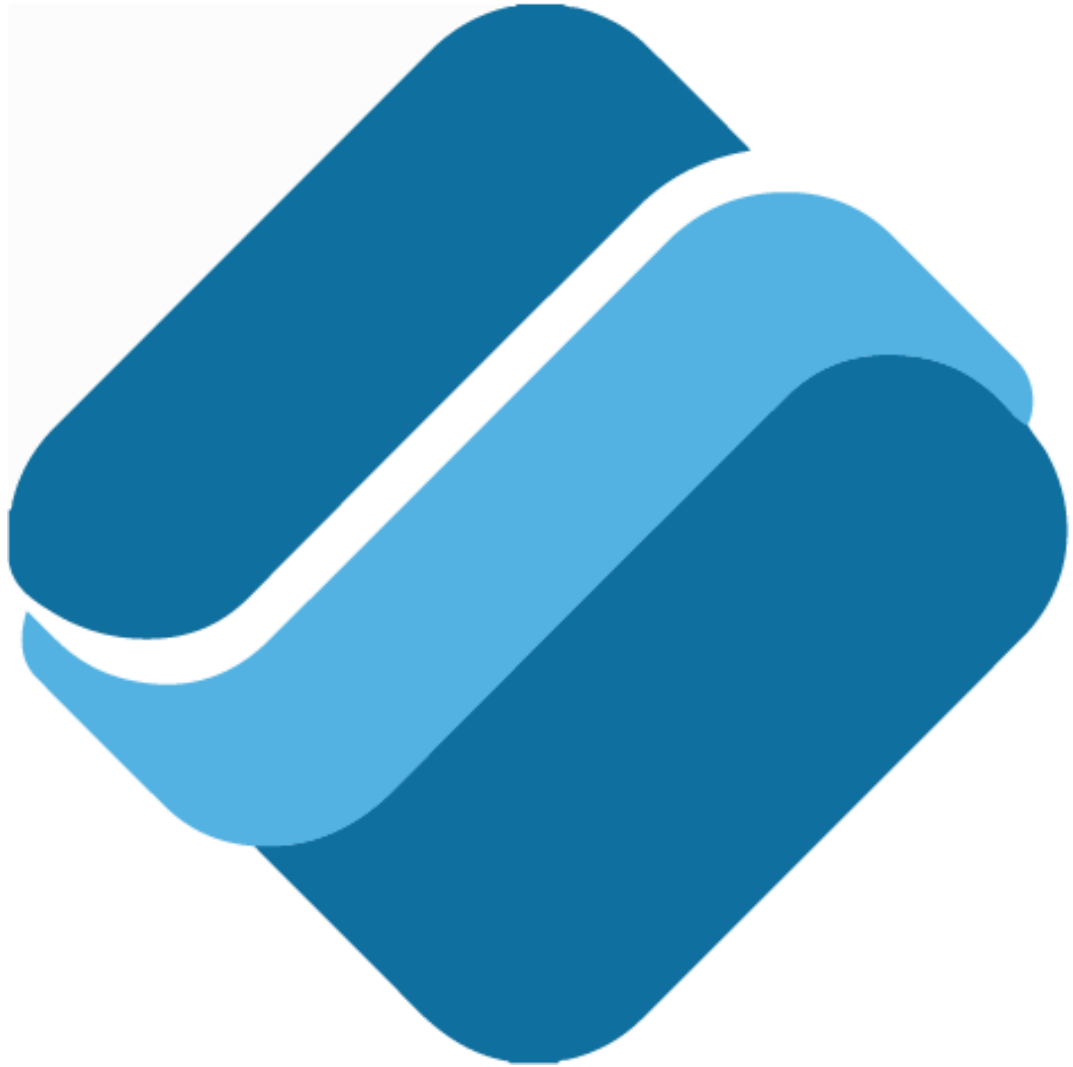
Pharming



Pharming Group NV

Sijmen de Vries
Chief Executive Officer

Investor Tour
Rotterdam

19 September 2018

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Agenda

19:00 uur - Receptie

19:25 uur - Welkomstwoord

19:30 uur - Korte corporate presentatie

20:00 uur - Sijmen de Vries Q&A met Leon Melens

20:30 uur - Audience Q&A

21:00 uur - Aanvang borrel

22:00 uur - Einde

Company Overview

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- Euronext: PHARM - market capitalization: ~€480 million (~$560 million) at €0.78 per share
- Headquarters in NL, R&D in France, EU and US commercial operations with approximately 170 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, Korea and Israel with other territories coming
  - Now being explored for larger indications: pre-eclampsia, CIN, Cardiac Protection, DGF
- Platform technology makes recombinant human molecules cleanly and efficiently
- New Enzyme Replacement Therapies (ERT) for other genetic conditions about to enter clinic

'We develop and commercialize human therapeutic proteins for innovative therapies meeting important unmet patient needs'

Corporate Highlights

RUCONEST® Commercialisation

- Re-acquisition of US commercialization rights from Valeant in December 2016
- H1 2018 revenues: €59.1 million
- Temporary supply issues at a major competitor during Q4 2017 now resolved

RUCONEST® Franchise Development

- Prophylaxis of HAE: CRL letter from FDA
- Ongoing investigator instigated studies
- Clinical trials for more convenient product formats to be initiated
- Clinical trial for additional large non-HAE-related indications to be initiated

Maturing pipeline beyond RUCONEST®

- Pompe program filing for IMPD early 2019, program for Fabry's disease to follow
- Use same transgenic founder technology to target $1 billion+ markets where all existing products have immunogenicity issues and boxed warnings

Financial Highlights

- Delivered net profitability for the half year 2018
- Revenues from product sales for the half year increased by 96% to €59.1 million (HY 2017: €30.1million), as a result of the continued increasing patient numbers
- Operating results improved by 288% to a profit of €16.3 million from €4.2 million in 2017, despite an increase in manufacturing and clinical activities related to the new indications
- The net result was a profit of €6.4 million (2017: loss of €30.2 million)
- Cash position at H1 2018: 66.9mil. (Year end cash position 2017: 60mil)

Three horizons of growth

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Making RUCONEST® a better HAE product

- Low volume IV
- Subcutaneous
- Intramuscular
- Painless intradermal
- Prophylaxis for HAE

Add more HAE sales

Meeting other unmet medical needs with the same product

- Pre-eclampsia
- Others such as Contrast-induced Nephropathy, Cardiac Protection, Delayed Graft Function and Hypovolemic Shock

Add more RUCONEST® sales

Meeting other unmet medical needs with another products

- α-glucosidase (Pompe)
- α-galactosidase (Fabry)
- Others

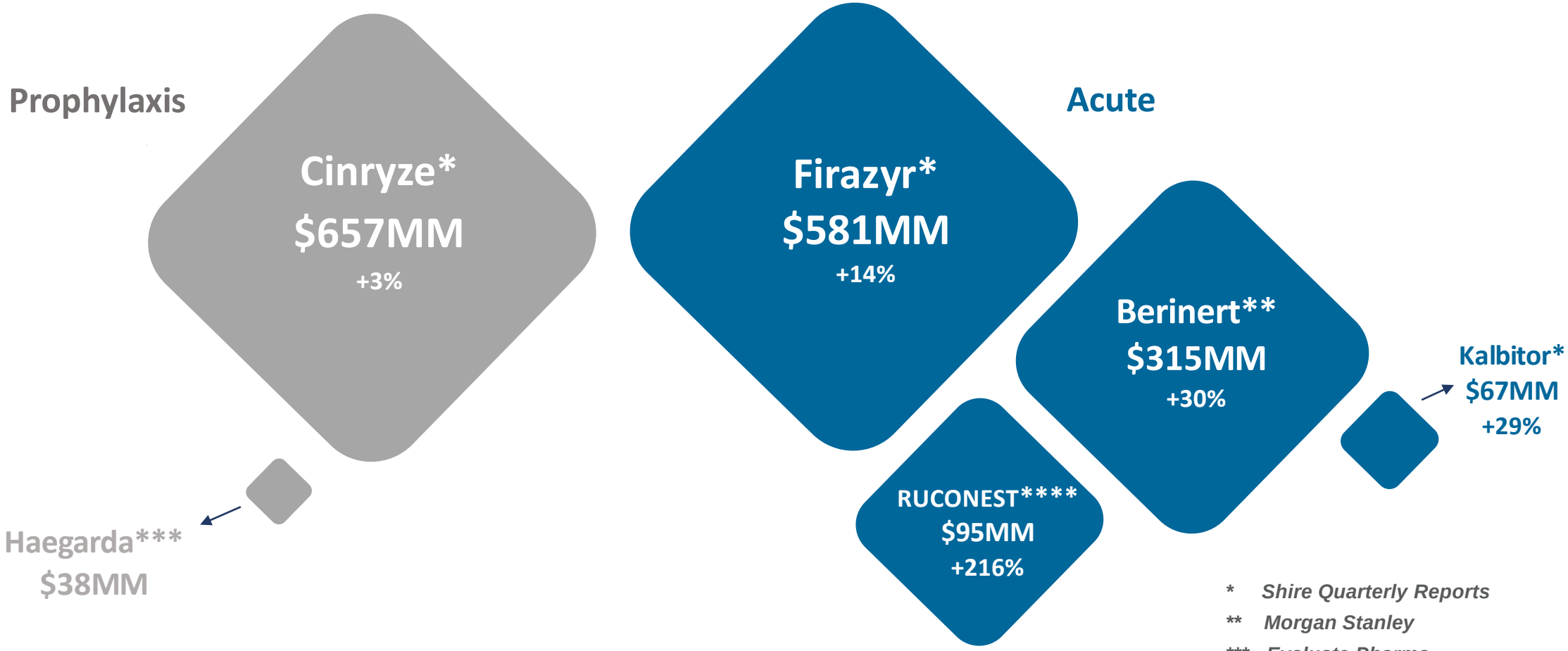
Add more products to sell

What is RUCONEST®?

- RUCONEST® = Recombinant human C1 esterase inhibitor (rhC1INH)
- C1 Esterase Inhibitor is a major part of the braking system for inflammation in the body
- RUCONEST® has an identical amino acid sequence to that of endogenous C1-INH in humans
- Same binding affinity to target-proteases and the highest purity of all available C1-INHs
- Some differences in glycosylation leading to faster clearance than plasma-derived C1-INH
  - No impact on clinical efficacy
- No risk of blood-borne pathogens
- EMA approval 2010, FDA approval 2014
- More than 70,000 post-marketing doses administered with no significant adverse events
- Easily scalable supply to match future demand; unlike plasma-derived C1-INH versions
- Strong know-how protection and data exclusivity until July 2026

US Market for HAE drugs

~$1.7 billion Sales in 2017 (Year-on-year growth 17%)



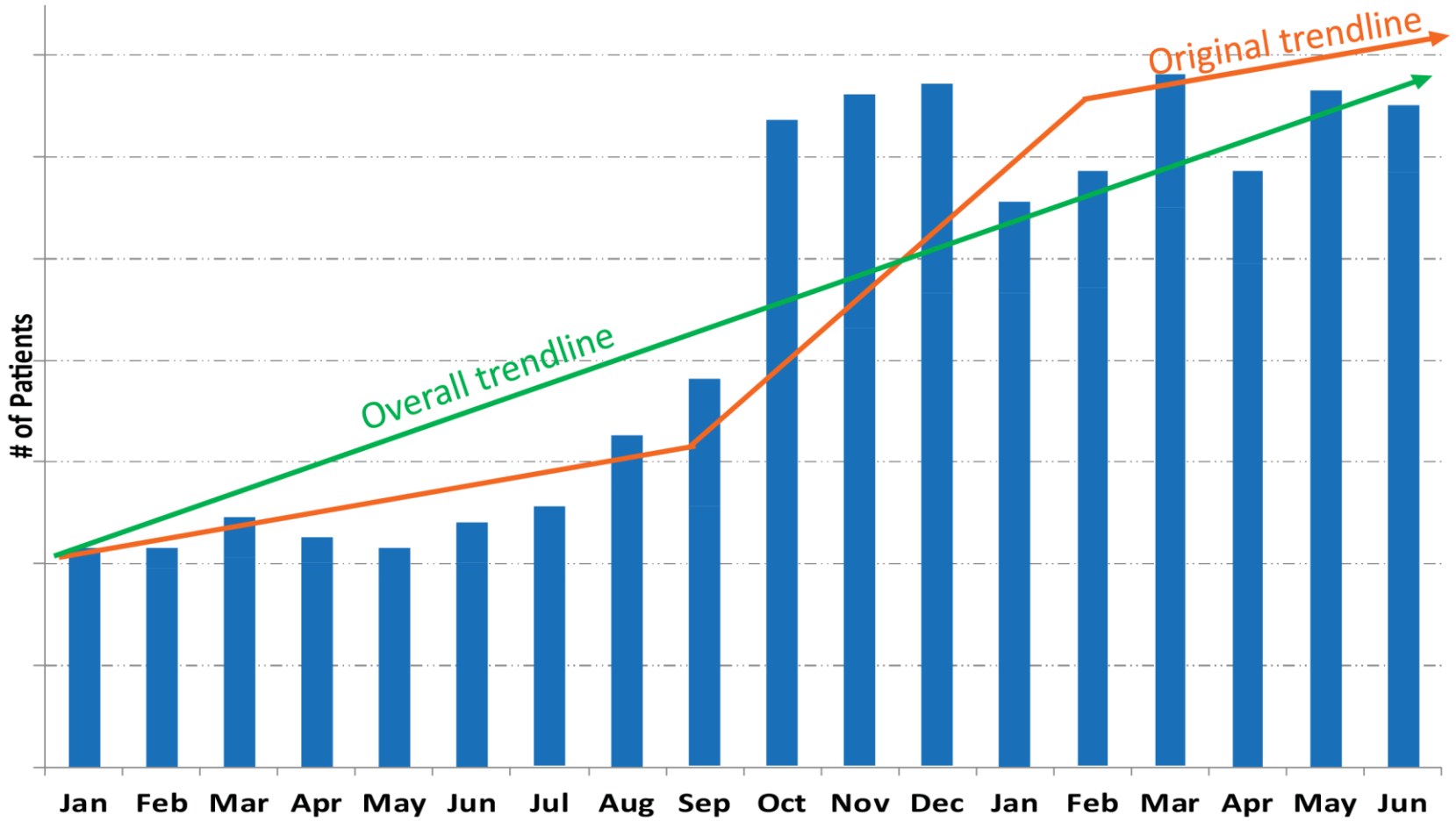
* *Shire Quarterly Reports*

** *Morgan Stanley*

*** *Evaluate Pharma*

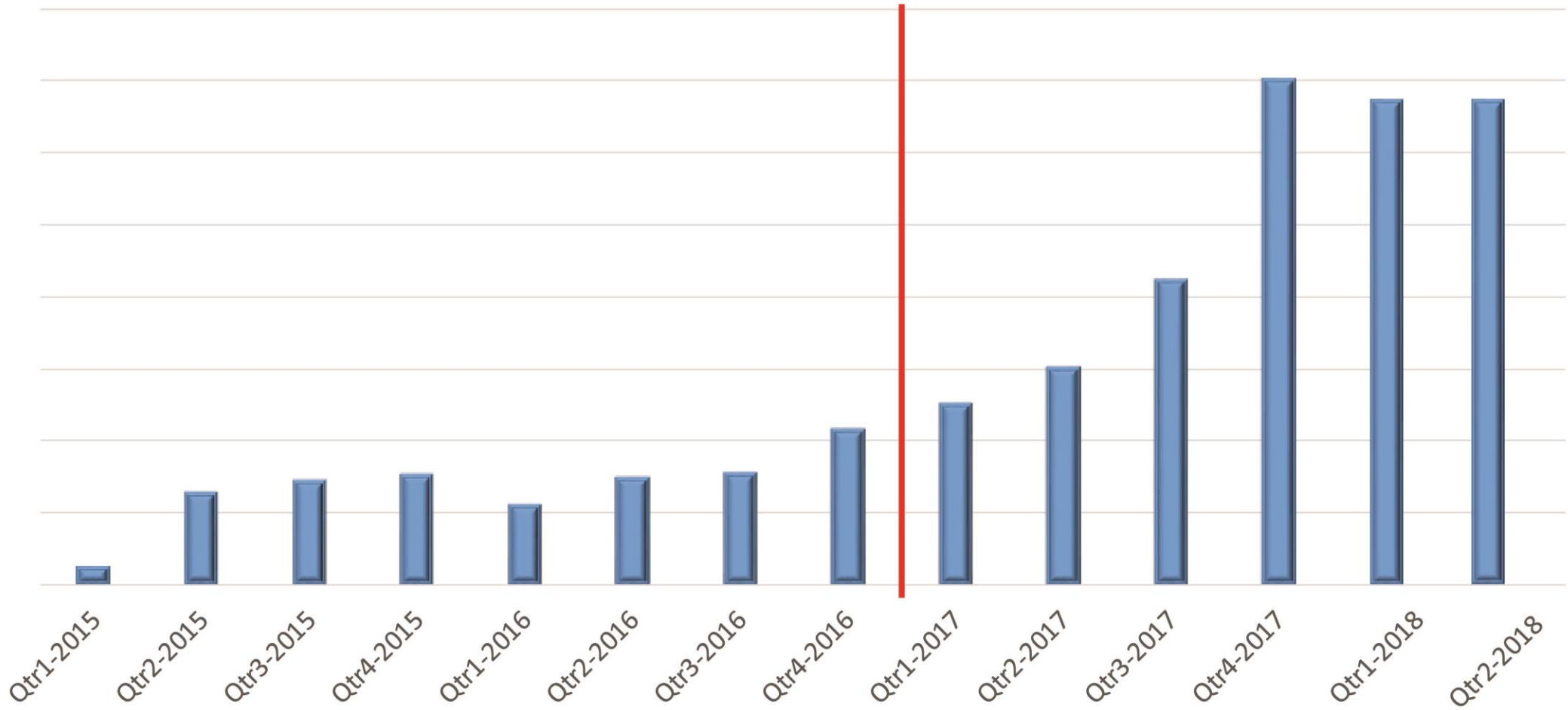
**** *Pharming Reported Net Sales*

Patient numbers following reacquisition of RUCONEST US rights

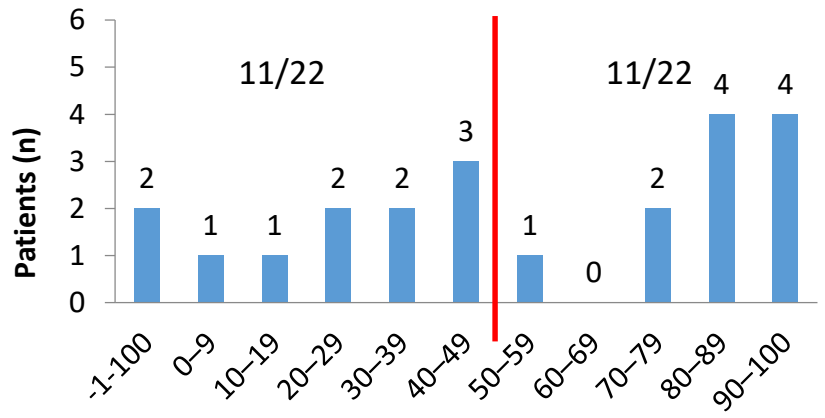


Data to 30 June 2018

US quarterly sales development in volumes

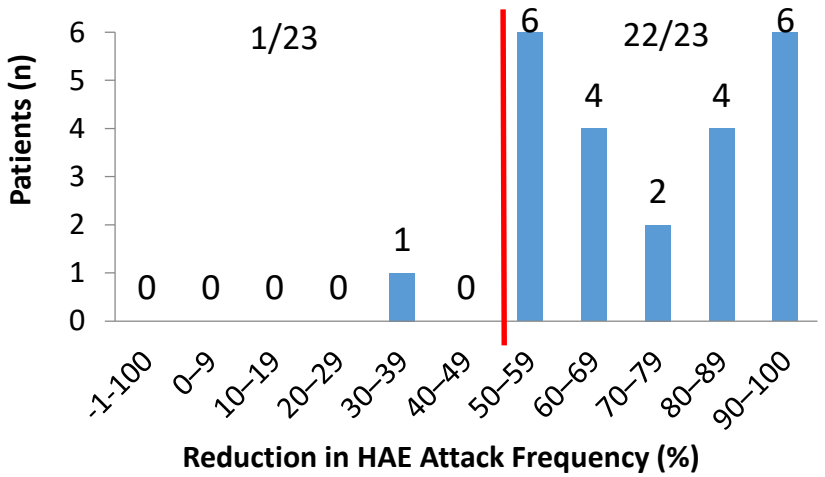


Comparing published results on HAE Prophylaxis



Cinryze® (1000 U Twice weekly)

- varying reduction of HAE attack frequency
- 12.7 attacks (placebo) vs 6.1 (Cinryze - pooled data across 12 weeks)
- 50% clinical response
- 52% reduction in attacks



RUCONEST® (50 U/kg Twice weekly)

- consistent reduction in attack frequency (n=23)
- 95.7% clinical response (all but one)* (PP)
- 74% reduction in attacks (PP)
- Heavily affected patients (average 7.5 attacks/month)

*Patients who had ≥50% reduction in the number of HAE attacks (normalized for the number of days the patient participated in the treatment period) from the placebo treatment period to the rhC1INH treatment period.

Source: Published data, US Food & Drug Administration

What patients want when living with HAE

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- Patients want a therapy that is convenient and pain-free

“My veins are shot, so I inject my medicine in my stomach. It is so painful and my stomach has bruises everywhere. I wish I had a medicine that didn’t hurt and was quick and easy to use.”

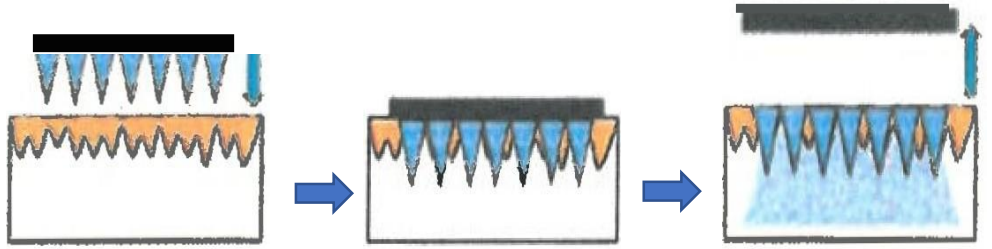
RUCONEST®: Developing better and painless formulations for HAE

Two low volume highly concentrated vials that could be used for:

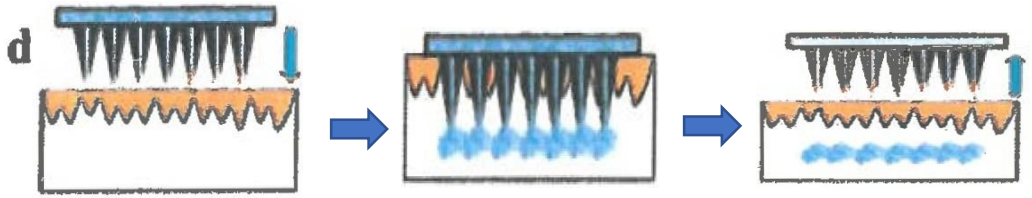
- Subject to regulatory approval: IV treatment of HAE attacks
- Clinical trial programs for intra-muscular (IM) treatment of HAE attacks and sub- cutaneous (SC) and intra- dermal (ID) prophylaxis of HAE attacks
- "RUCONEST® Liquid": ready to use 3 ml vial containing > 500 U/ml; in technical development
- "RUCONEST®lite": vial containing 2100 Units lyophilized rhC1INH to be dissolved in 3 ml water
  - Fast reconstitution time: 3 minutes (5-6 minutes for normal RUCONEST®)
- Required clinical studies for IM and SC/ID applications are being designed

New intradermal formulations with RUCONEST®

- The “RUCONEST® liquid” formulation can be used as starting material for the generation of intradermal application systems
- New proprietary ‘painless’ intradermal delivery applications are being developed:
- Dissolving point device:

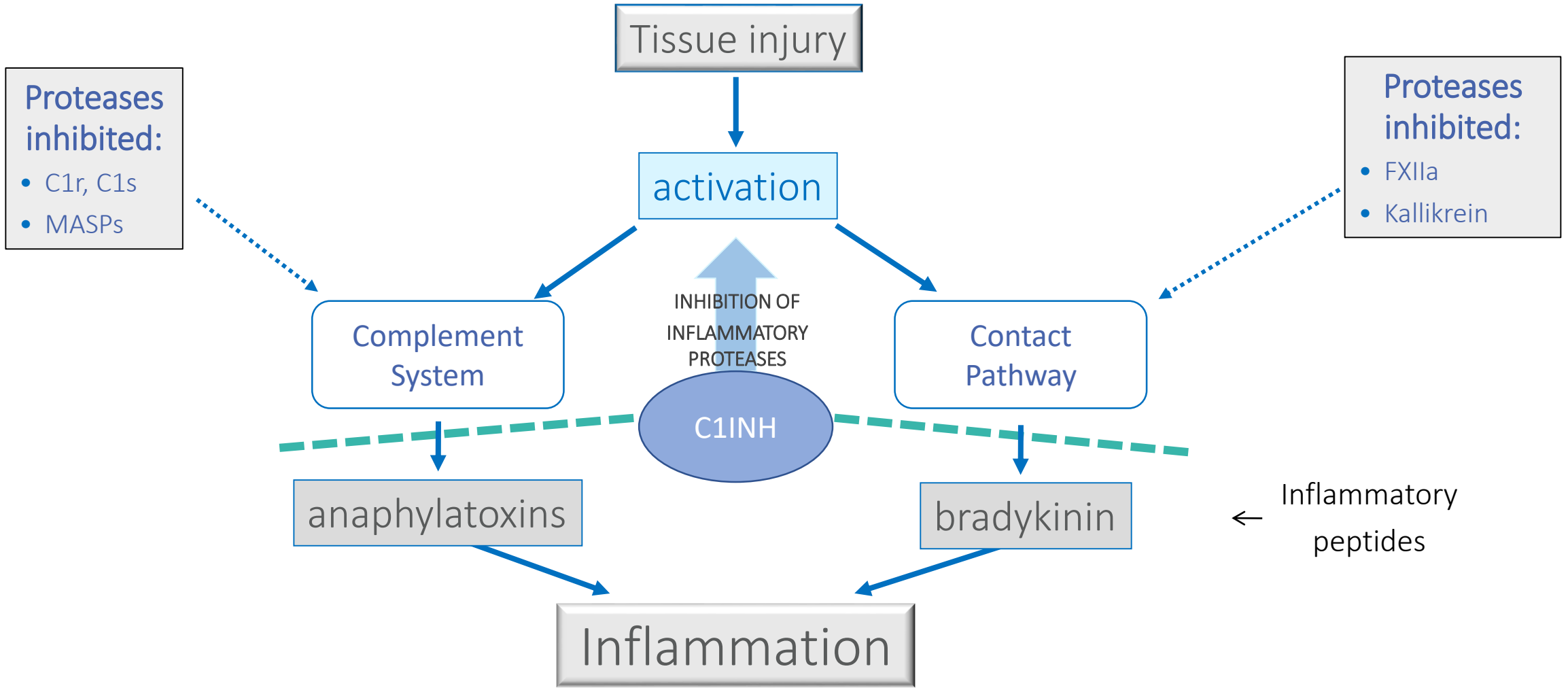


- Reservoir device:



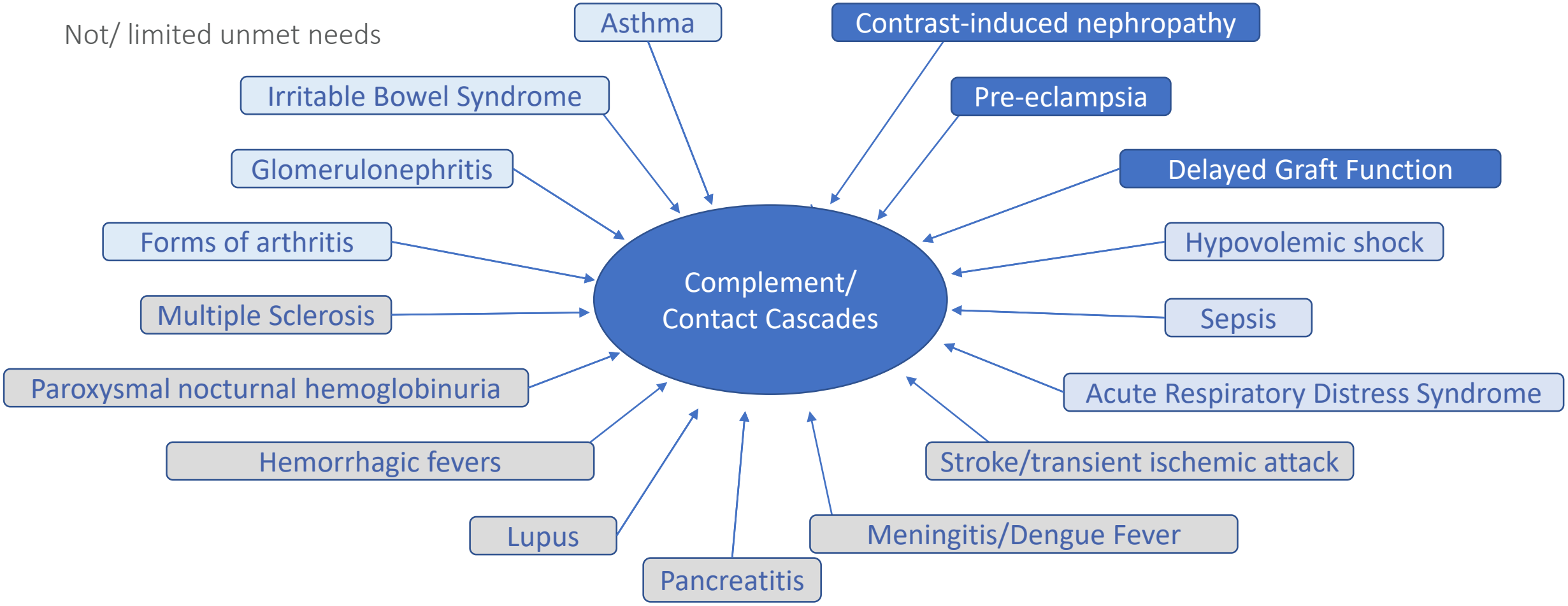
- These painless versions should differentiate RUCONEST® from competitors, all of whom have painful injections

C1INH : Multiple Anti-inflammatory Effects



Other potential options for development of rhC1INH

The complement and contact systems are known to play a role in many diseases with an immune component, such as:



New Activities with rhC1INH

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Initial Therapeutic Indications selected:

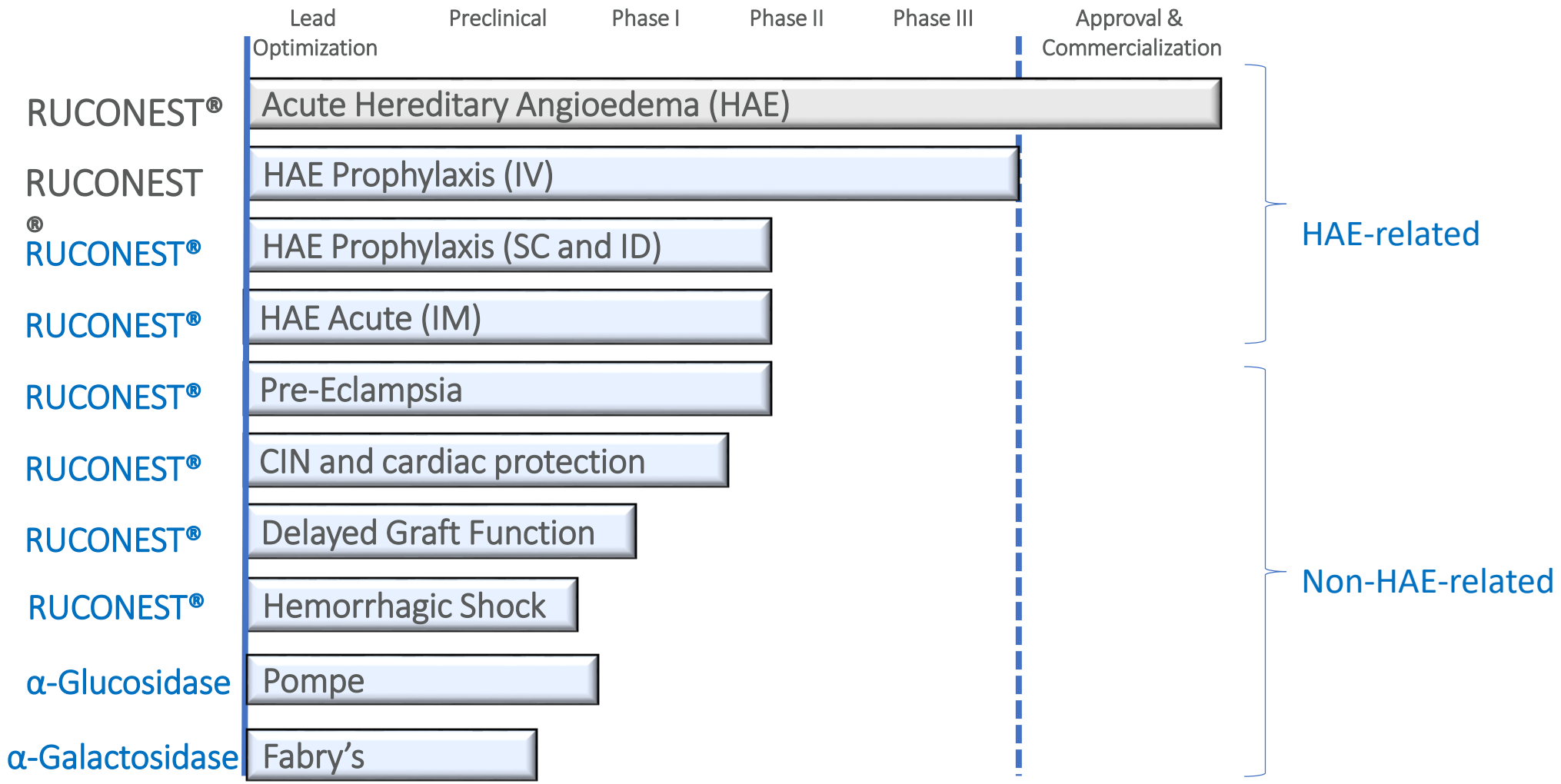
New Potential Indications using existing formulation

- Tissue Damage after Toxic Event :- Pre-Eclampsia (new Pharming)
- Tissue Damage after Hypoxic Event :- Delayed Graft Function (new Investigator initiated study)
- Organ damage after contrast media application:- Contrast-induced Nephropathy (ongoing Investigator initiated study)
- Vascular/cardiac damage due to investigation/operation:- Cardiac protection (depends on data from above study)
- Shock response after trauma:- Hypovolemic Shock – ongoing preclinical research projects with US Army and US Air Force

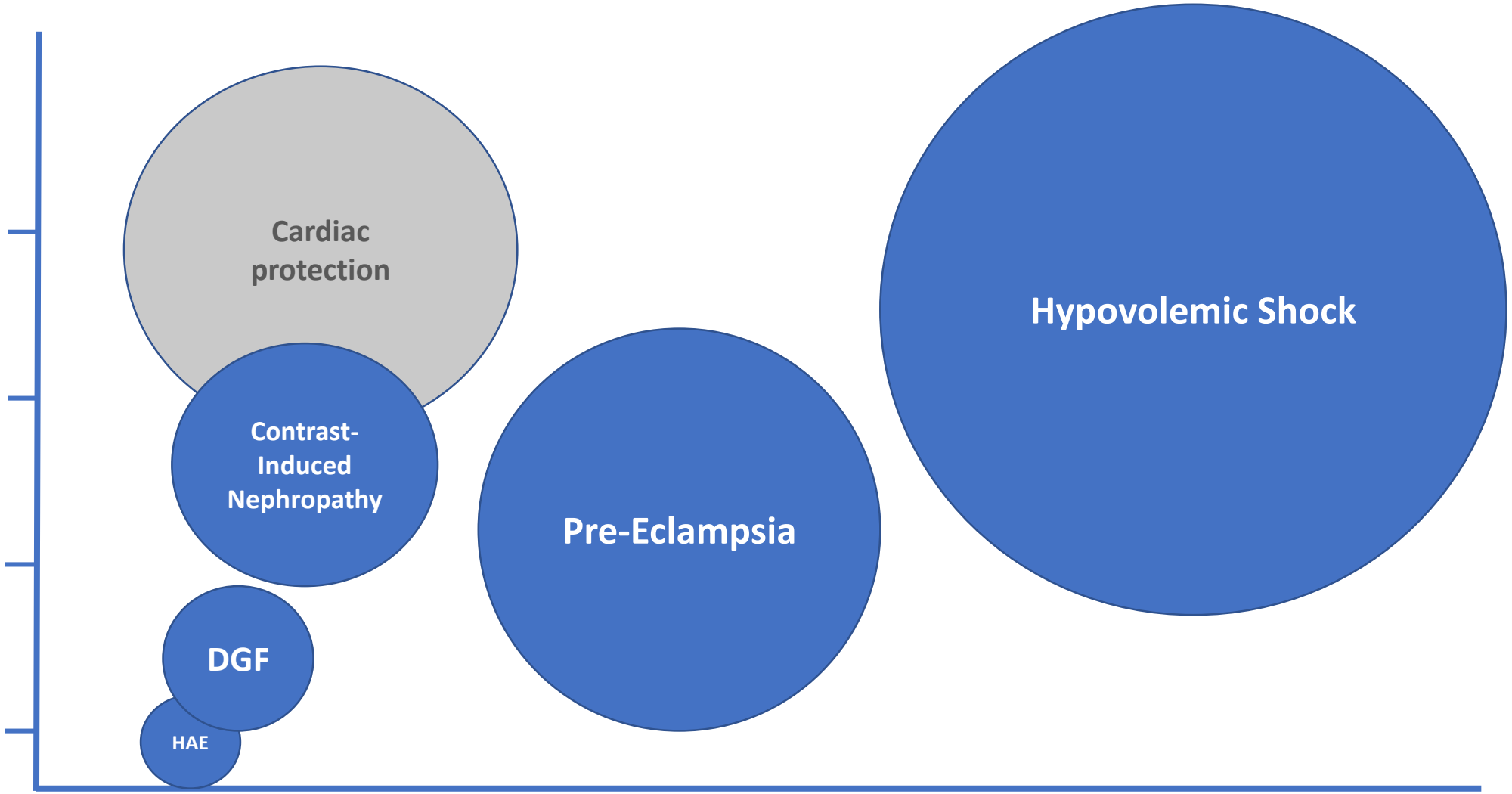
Brand New IP:

- New Pharming patents filed in 2018 covering the new indications
- Patents cover all forms of rhC1INH

Expansion of pipeline to multiple products and markets



Potential addressable future markets

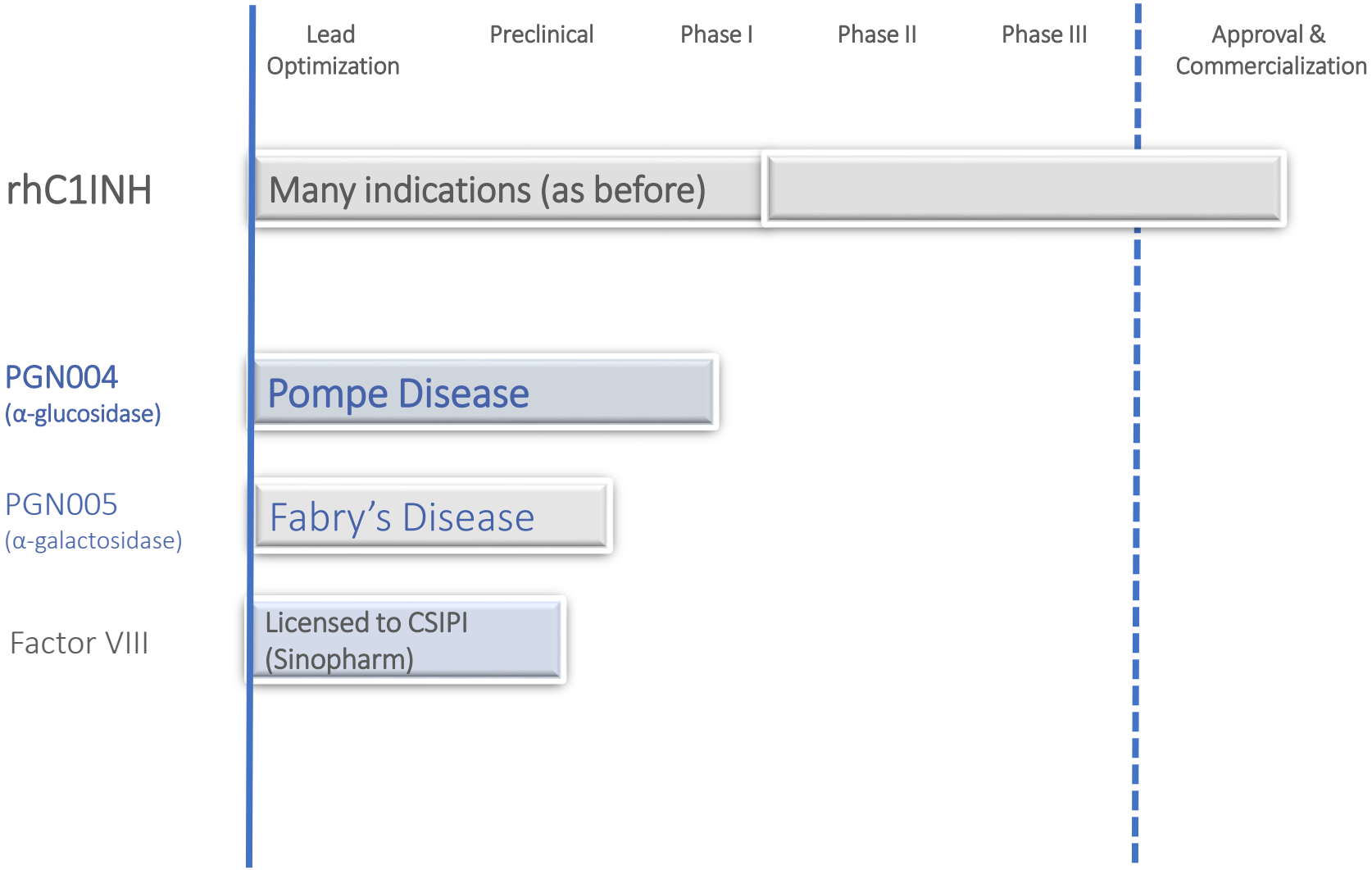


Not to scale!

Expansion of production capacity

- The rabbit-based production of rhC1INH is very scalable; up-scaling can be executed rapidly
- To serve future potential large indications in a more economical way, Pharming will restart its previously-developed and characterized cattle-based production lines of rhC1INH
- Clinical programs could be initiated with RUCONEST and switched to cattle version during program following conversion studies
- Cattle-derived rhC1INH may have some benefits over the current rabbit version, including an extended serum half-life as result of an improved (even closer to human) glycosylation pattern
- New IP to be filed on the cattle-derived rhC1INH

Expansion of Pharming to a Multiple Product Franchise



Pompe and Fabry's diseases

α-glucosidase and α-galactosidase

- Market potential for Pharming is over $1 billion per year in each indication
- Attractive market:
  - All current products have severe shortcomings and boxed warnings, but together sell for over $1 billion
  - Second generation products have their own shortcomings
  - Many patients are not on therapy because of antibody formation or adverse reactions
- α-glucosidase for Pompe disease now finalising last parts of manufacturing file and upscaling production to produce clinical trial material
- IND expected to begin 1H2019
- α-galactosidase for Fabry in mid preclinical development; expected to reach IND filing stage in 2020

rh-α-glucosidase (rhaGLU) for Pompe

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Pompe's disease

- Rare autosomal recessive lysosomal storage disease
- Caused by the lack of functional α-glucosidase (aGLU or GAA)
- 5-10k patients world-wide, with global market over $1 billion
- Usually fatal in the first year of life if untreated, can still be fatal if diagnosed later

rhaGLU

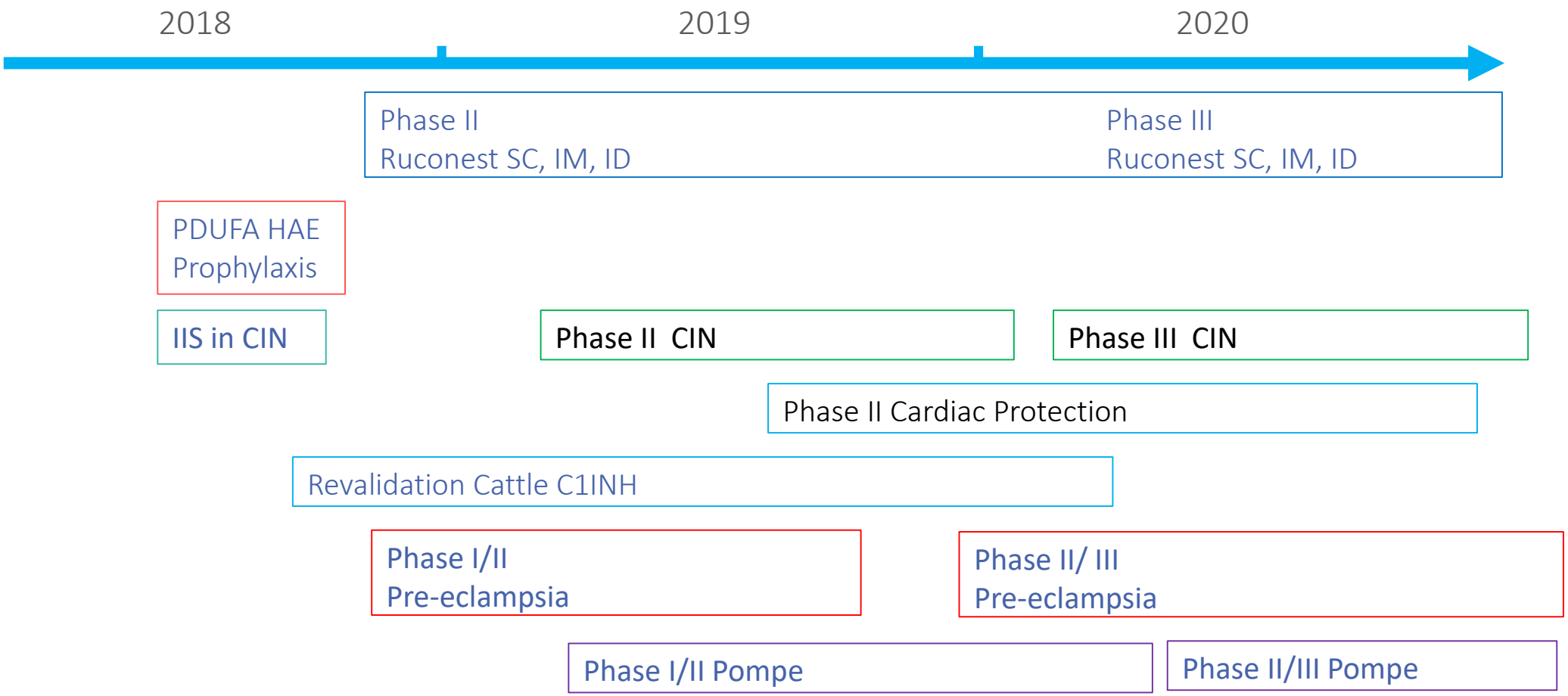
- Risk/ benefit profile of existing products is poor, with limited penetration of the population as a result
- Boxed warnings for immunogenicity / antibody formation and associated sub-optimal clinical results
- Cell line-derived recombinant versions of highly glycosylated proteins such as rhaGLU and rhC1INH appear to reach "the limits" of capabilities of cell-based reactors, with products usually highly immunogenic or having off-target effects

rh-α-glucosidase (rhaGLU) for Pompe

- rhC1INH RUCONEST (equally highly-glycosylated) from our transgenic (rabbit) platform does not generate relevant antibody responses
- A small 36-week clinical trial in infants with previous transgenic (rabbit-derived) rhaGLU showed good efficacy and did not report any safety concerns (2001)*
- De novo proprietary constructs for our rabbit platform for rhaGLU have been developed and a new recombinant rhaGLU is being produced for initial clinical trial supplies
- New version is closer to natural human GAA than previous Pharming version, and also believed to be much closer than any other tested recombinant version to date

Potential for significant newsflow over the coming years

Assumed solely for purpose of diagram: positive results of studies



12 Month Outlook

- Continued growth in sales of RUCONEST® driven by the US and EU operations
- Continuation of positive trend in operating results
- Continuation of positive Net Earnings during the year
- Continued investment in the expansion of production of RUCONEST
- Research and (Clinical) Development investments:
  - RUCONEST® in HAE (SC/ID/IM) with low volume vial trials
  - Clinical trials for additional indications for RUCONEST®
  - New pipeline: Clinical development Pompe disease early 2019
- Increasing marketing activity, such as opening new countries for RUCONEST®
- Continue to support all our marketing partners to maximize the sales and distribution potential of RUCONEST® for patients in all territories

Q&A with Leon Melens

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Questions?

Sign up for our press releases, or ask further questions
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