



Een terugblik: Pharming Group

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- Financiële resultaten van het derde kwartaal, waar staat wij nu?
- “Basel Studie”: Positieve studieresultaten van eerste door onderzoekers geïnitieerde in contrast-geïnduceerde nefropathie
- Recente Investor tour 2018
- Ruconest nieuwe toedieningen
- Nieuwe therapieën – Pompe
- Verwachte nieuwsflow

First 9 months 2018: Financial Results



9 months to 30 September

Amounts in €m except per share data	2018 3 rd Quarter	2018 1 st 9 months	2017 1 st 9 months	% Change
Income Statement				
Revenue from product sales	38.6	97.7	56.0	74%
Other revenue	0.2	0.6	0.7	(14%)
Total revenue	38.8	98.3	56.7	73%
Gross profit	32.4	82.4	48.8	69%
Operating result	14.7	31.0	12.7	144%
Net result	5.4	11.7	(37.7)	131%
Balance Sheet				
Cash & marketable securities	72.2	72.2	38.6	87%
Share Information				
Earnings per share (€): - Undiluted	0.009	0.019	(0.077)	125%
- Fully diluted	0.008	0.017	n/a	

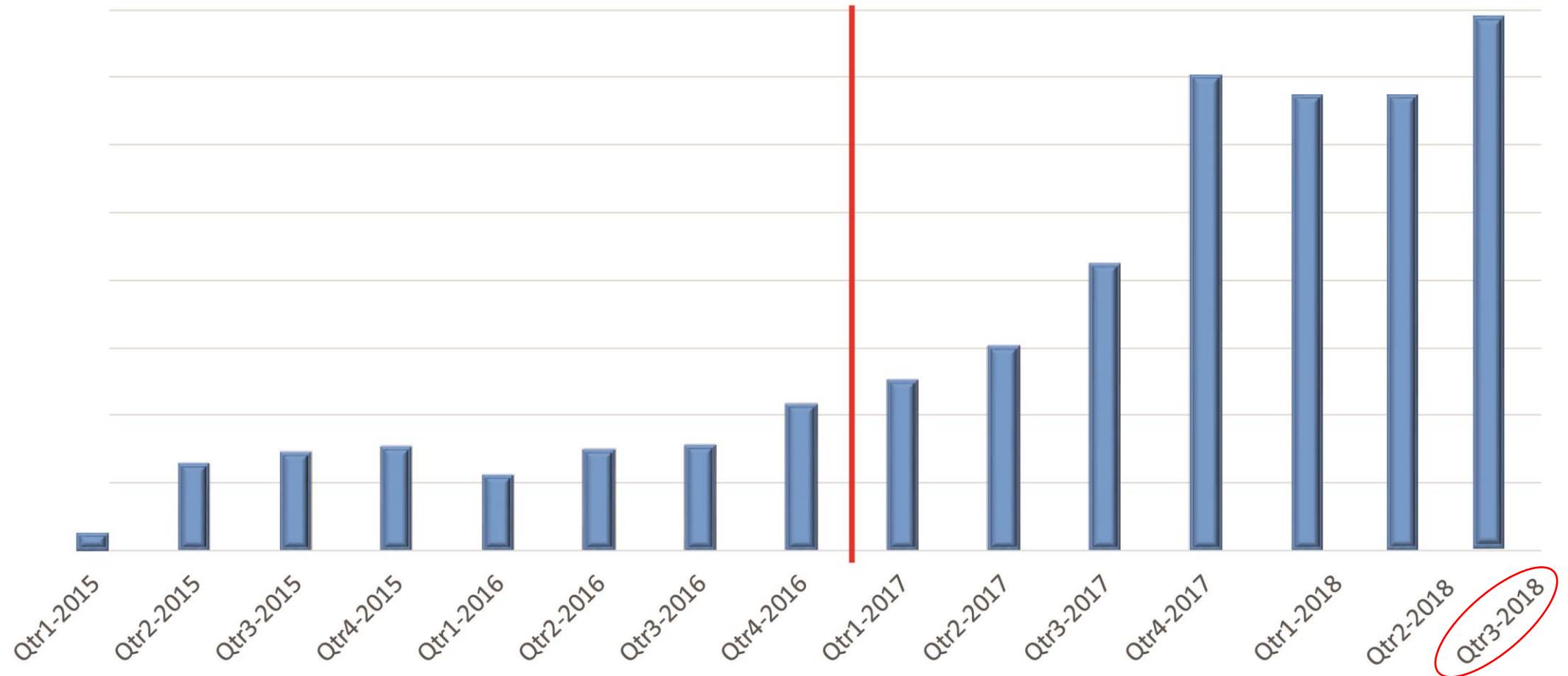
Compared with the first nine months of 2017 (on a like-for-like basis):

- Product revenues up 74% to €97.7 million, operating profit up 144% to €31.0 million, net profit up 131% to 11.7 million

Compared with the last quarter ended 30 June 2018:

- Product revenues up 30% to €38.6 million, operating profit up 82% to €14.7 million, net profit up 77% to €5.4 million
- Cash increased to €72.2 million (after €7.5m repayment of debt) to invest in key growth drivers

US quarterly sales development in volumes



Update: Contrast-induced Nephropathy (CIN)



Basel Study results:

Positive results from a Phase II investigator-initiated study of RUCONEST®

- RUCONEST® showed a statistically significant effect ($p=0.038$) in reducing Neutrophil Gelatinase-Associated Lipocalin (NGAL).
- The results were especially clear in the sub-group of patients ($n=30$) undergoing PCI. The intent-to-treat analysis in this group showed that patients on RUCONEST® had a median increase in peak urinary NGAL concentration within 48 hours of 1.8 ng/ml compared with an increase of 26.2 ng/ml in the placebo arm ($p=0.04$).
- This corresponds to a clear difference in the median percentage change in the peak urinary NGAL level within 48 hours of 11.3% in the RUCONEST® arm and 205.2% in the placebo arm ($p=0.001$).
- Overall patients undergoing invasive procedures requiring high volumes of contrast media experienced a stronger benefit from the RUCONEST® treatment.

Next steps:

- Following positive results, we will continue discussion with Dr. Osthoff and other experts with the aim to perform further clinical development to establish efficacy and efficiency of RUCONEST® treatment in patient groups likely to experience the greatest benefit.

Investor Tour 2018



Three horizons of growth

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



Add more RUCONEST® sales

Meeting
other unmet
medical needs
with another
products

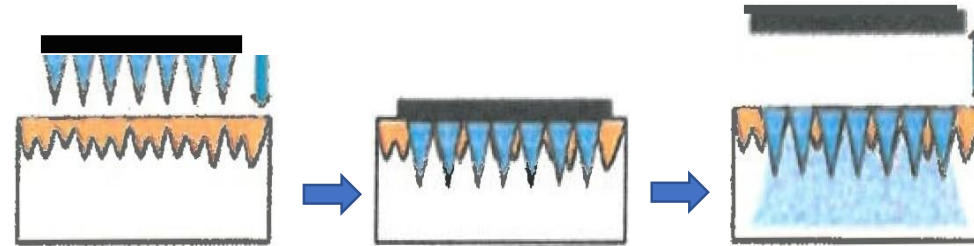
- α -glucosidase (Pompe)
- α -galactosidase (Fabry)
- Others



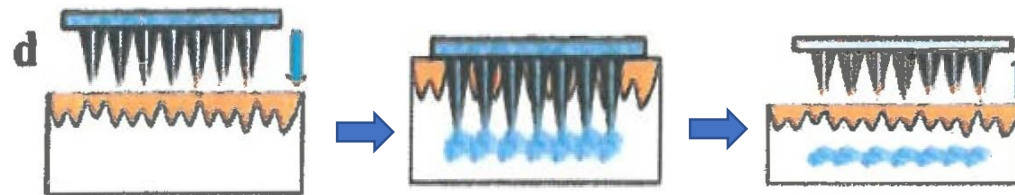
Add more products to sell

- 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 whom have painful injections

New Activities with rhC1INH (RUCONEST®)



Initial Therapeutic Indications selected:

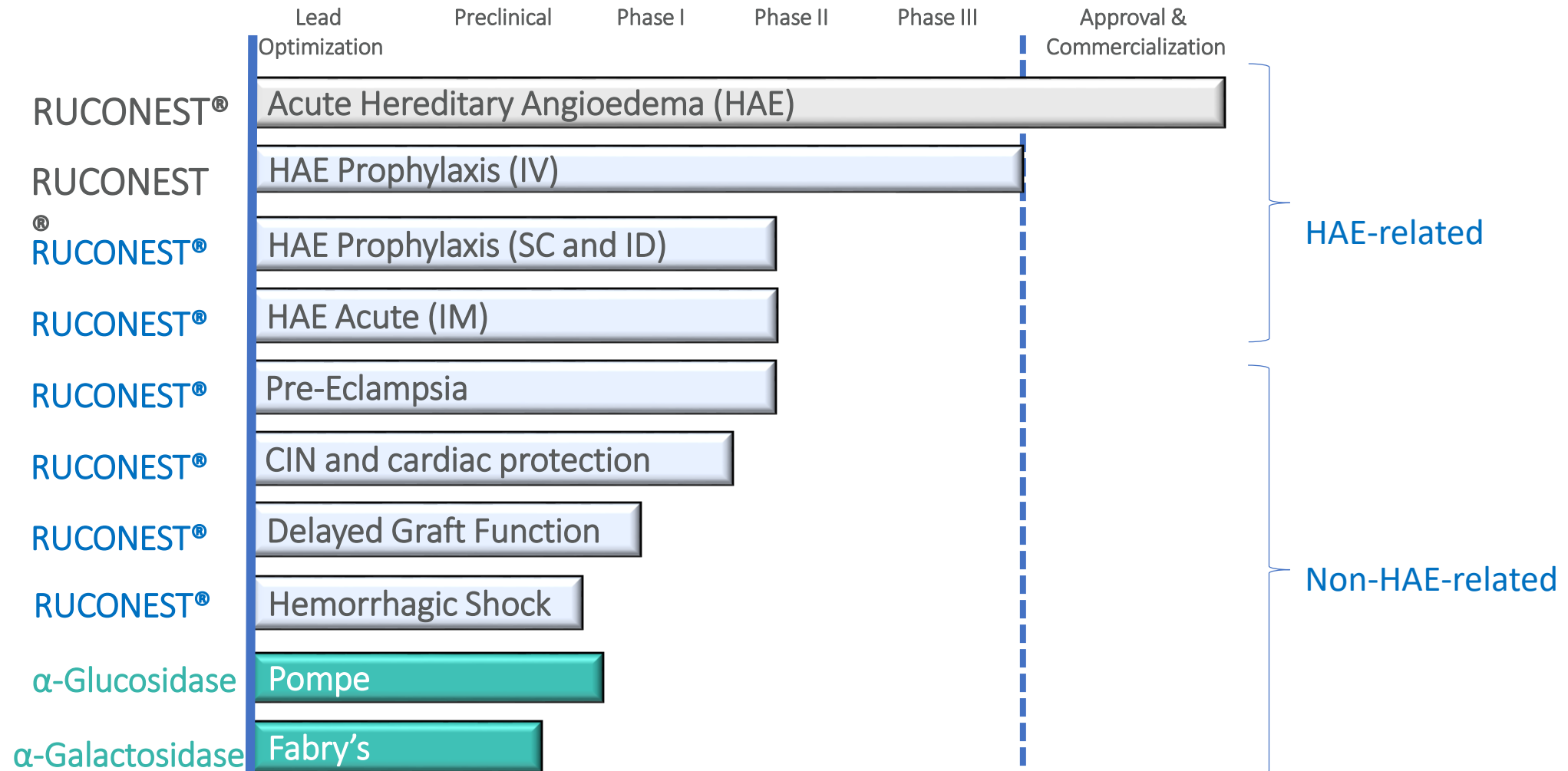
New Potential Indications using existing formulation

- Organ damage after contrast media application:- **Contrast-induced Nephropathy (CIN)**
- Tissue Damage after Toxic Event :- **Pre-Eclampsia (Zwangerschapsvergiftiging)** (new Pharming)
- Tissue Damage after Hypoxic Event :- **Delayed Graft Function** (new 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:

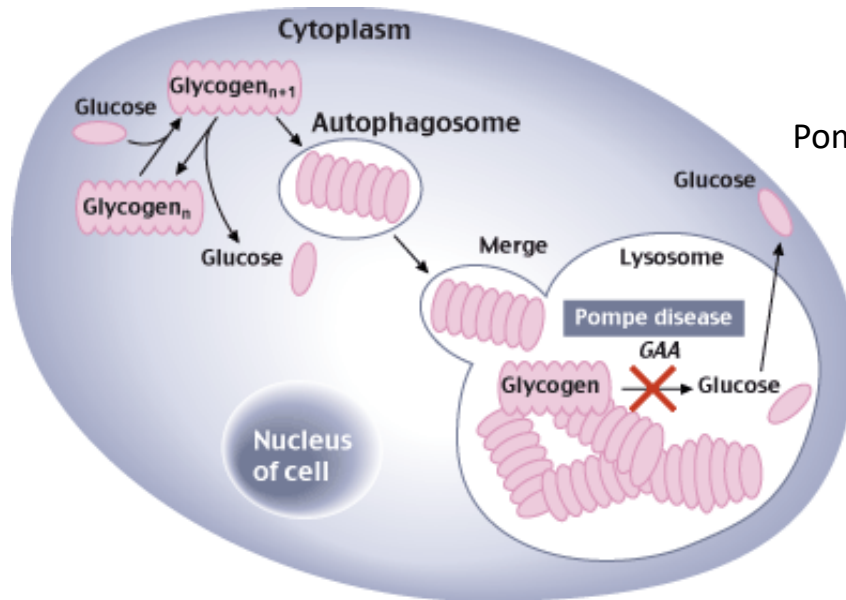
- New Pharming patents filed in 2018 covering the new indications
- Patents cover all forms of rhC1INH

Expansion of pipeline to multiple products and markets

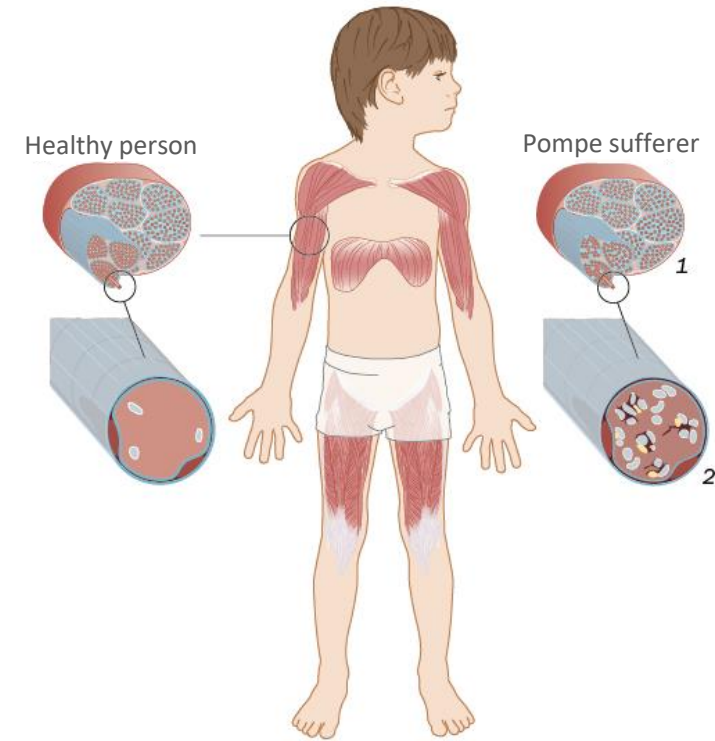


Pompe diseases

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

α -glucosidase

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**
 - 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)*




α -glucosidase

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 \$\alpha\$ -glucosidase](#) than previous Pharming version, and also believed to be much closer than any other tested recombinant version to date
- 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

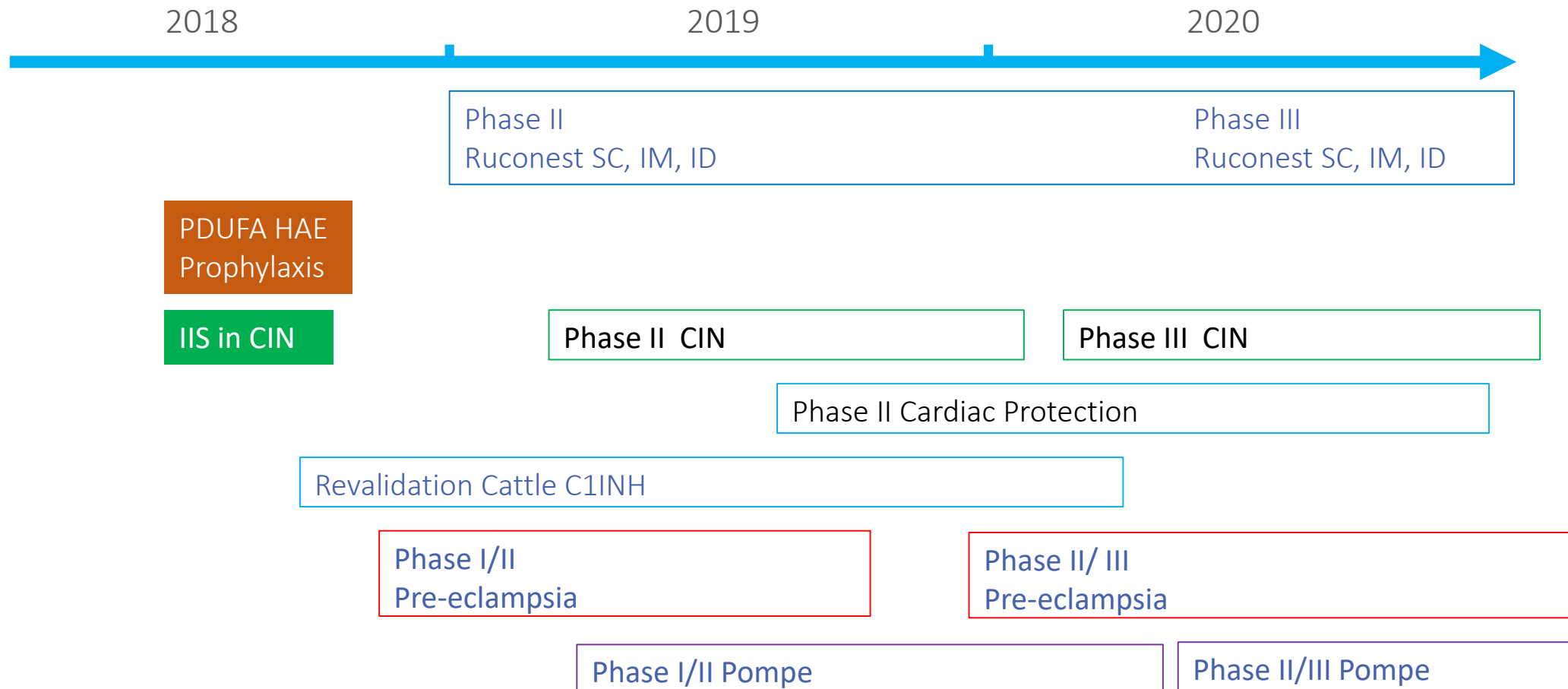


- FY 2018 revenues from product sales to be in the range of most analysts' forecasts and for the fourth quarter results to be in the same range as the third quarter, driven by continued underlying demand balanced by increasing competition
- Achievement of a continued positive net result, continued operating profit and positive cashflows for the remaining quarter
- Continued investment in the production of RUCONEST® in order to ensure continuity of supply to the growing markets in the US, Europe and the RoW
- Approval for the pre-eclampsia study and commencement of that study
- Continued and enhanced support for patients in all territories, as we continue to believe that RUCONEST® represents a fast, effective, reliable and safe therapy option for all HAE patients no matter their situation
- Continued progress in the new pipeline programs in Pompe disease and Fabry's disease, and additional development opportunities and assets as they occur



Increasing
sales and continued
positive results

Assumed solely for purpose of diagram: positive results of studies



Questions?



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Tickers:
ENXTAM: PHARM
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