Pharming Group N.V.

Sijmen de Vries

Chief Executive Officer

NL investor Tour 2019

Leiden, Eindhoven, Rotterdam, Utrecht

November- December 2019

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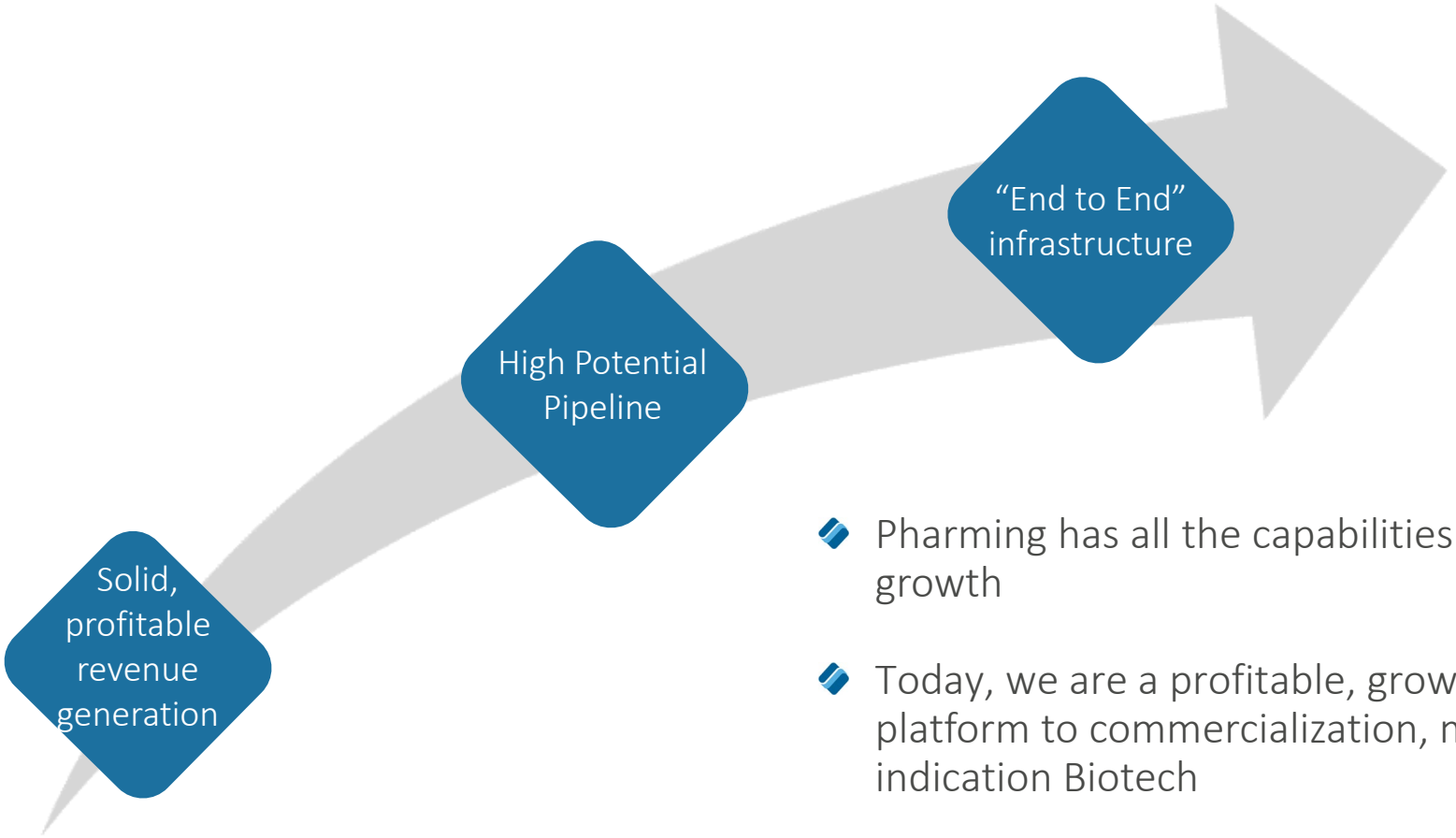
Company Overview

- **Public Company:** Euronext: PHARM: ~€900m- 1bn (~$1-1.1 bn)
- **Located:** the Netherlands, ~210 employees globally
- **Current Focus:** Rare and Ultra-rare disease development and commercialization
  - Marketed product: **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
- **Profitable and cash flow positive with 9M2019 net sales of €123M and expecting continued growth in sales**

Pharming today and into the future



- Pharming has all the capabilities needed for sustainable, high growth
- Today, we are a profitable, growing and fully integrated, from platform to commercialization, multiple product, multiple indication Biotech
- From this we can drive lasting additional growth by developing innovative solutions in select rare, ultra-rare and specialty diseases

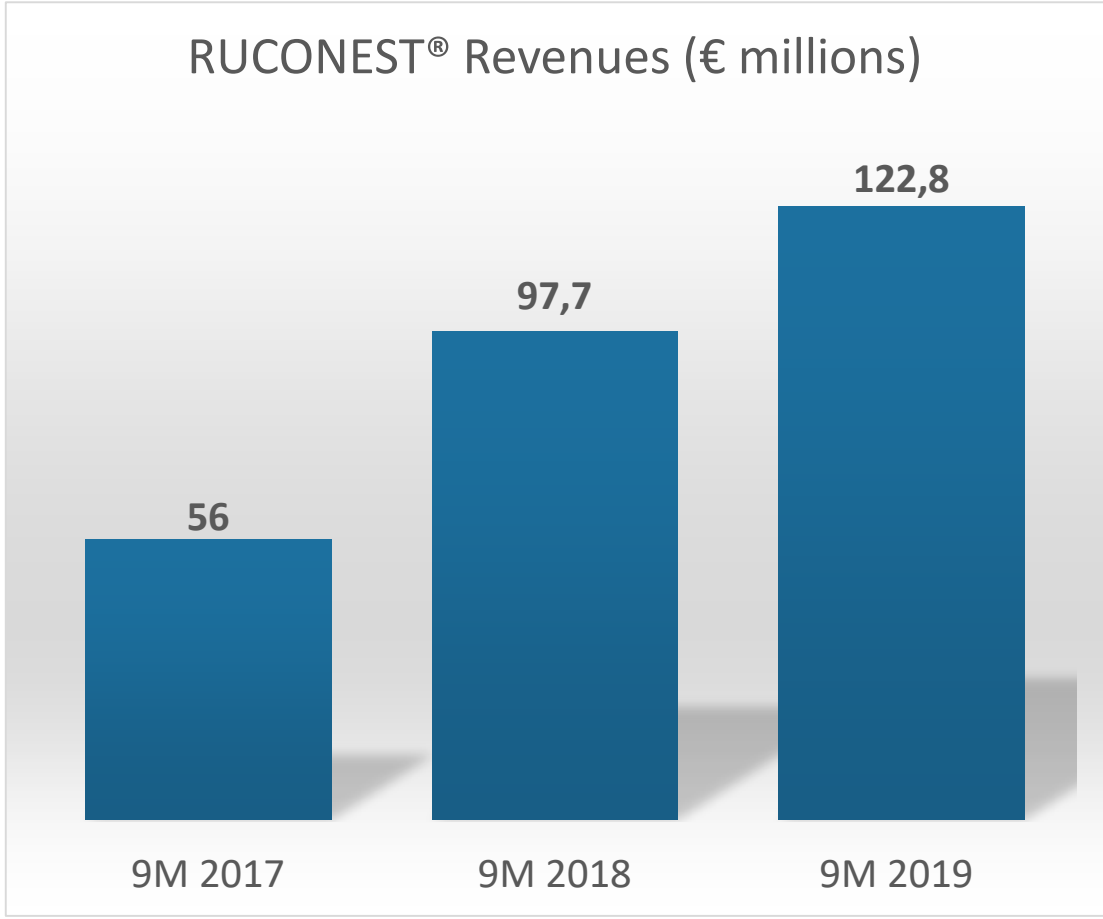
Pharming

Solid, profitable revenue generation

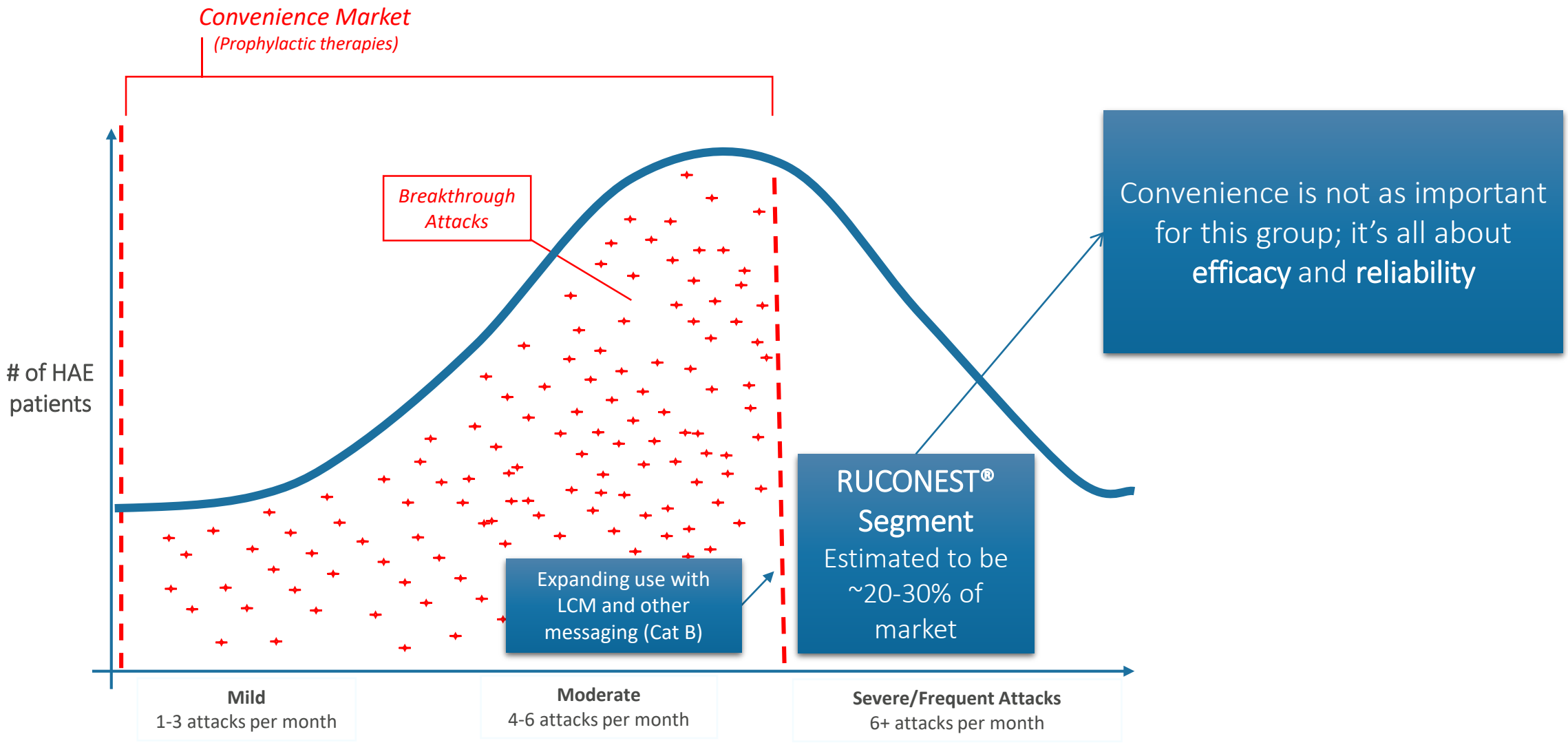
RUCONEST® : Strong Execution of Commercial Strategy

- HAE is a complex, serious disease with many idiosyncrasies and a varied market. The current approved therapies all address certain specific segments/phenotypes of HAE.
- RUCONEST® as the only recombinant PRT serves a segment the other therapies are unable to serve in an adequate way, due to its dosing and method of administration
- Pharming, as a result of the solid RUCONEST®; business, has a strong balance sheet with growing cash position
- With Q3 results, we are almost at the full year 2018 level

Pharming is in a very strong position to execute and grow



RUCONEST®: Patient Segmentation

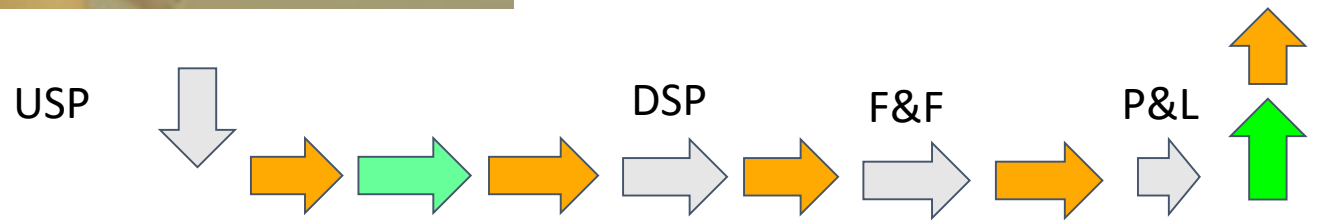


1. Banerji A, et al. Allergy Asthma Proc. 2018;39:212-223

High Potential Pipeline

From Milk to Medicine

Pharming



Production

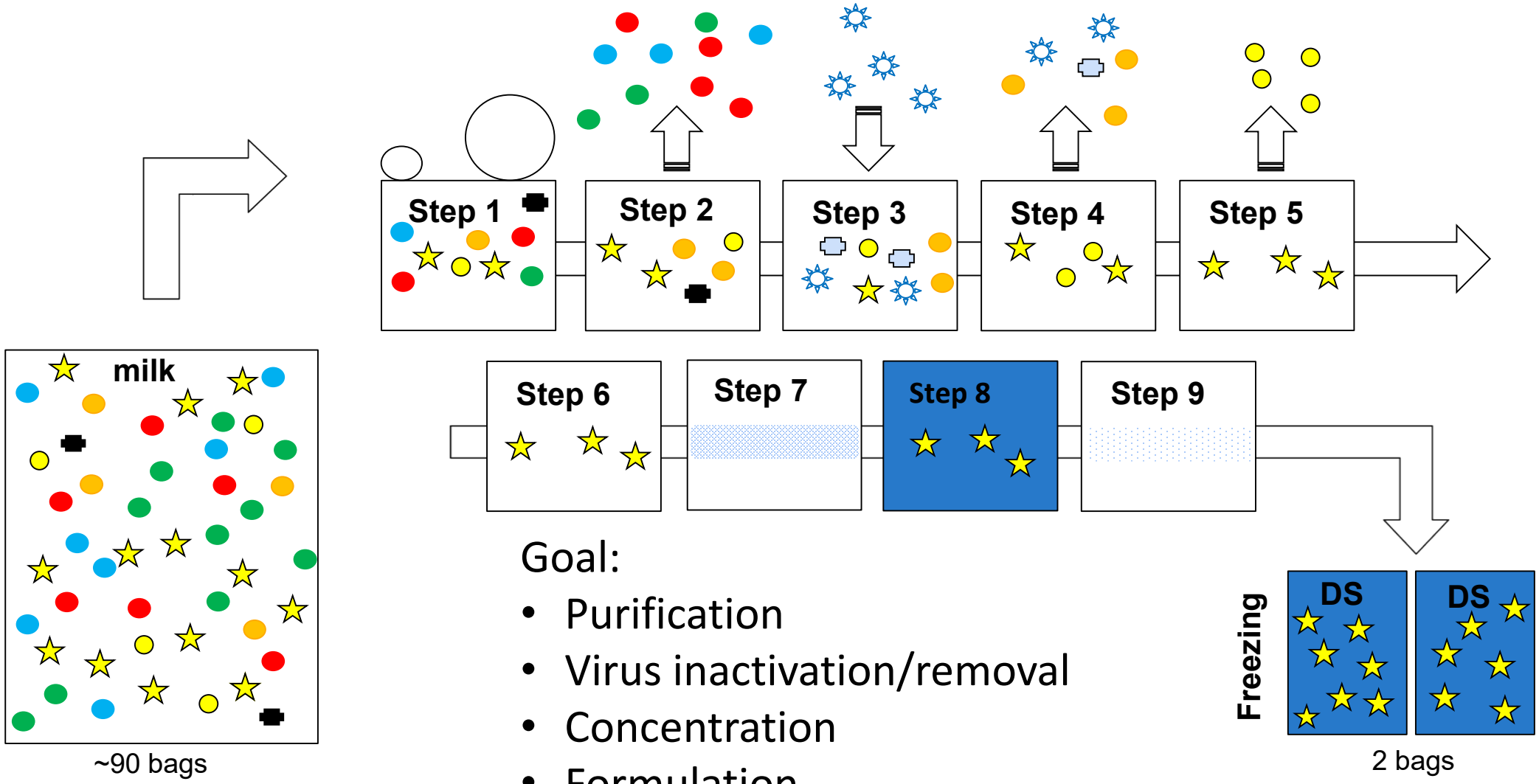
Transport

Storage

11 steps!

Approx 9 months!

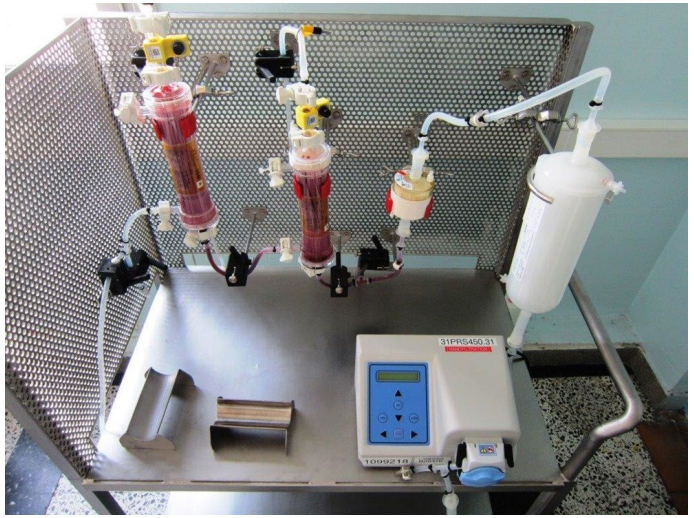
Step 5: Downstream Processing



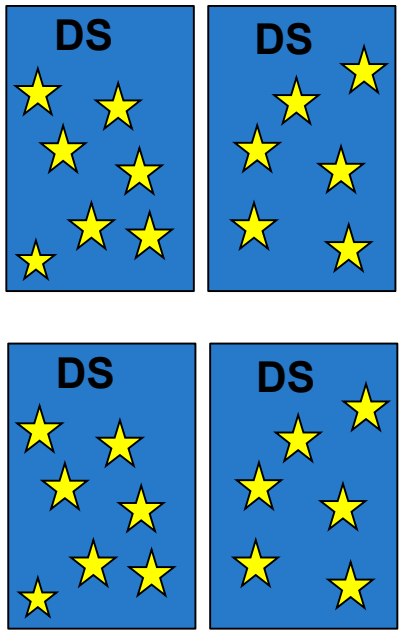
Goal:

- Purification
- Virus inactivation/removal
- Concentration
- Formulation

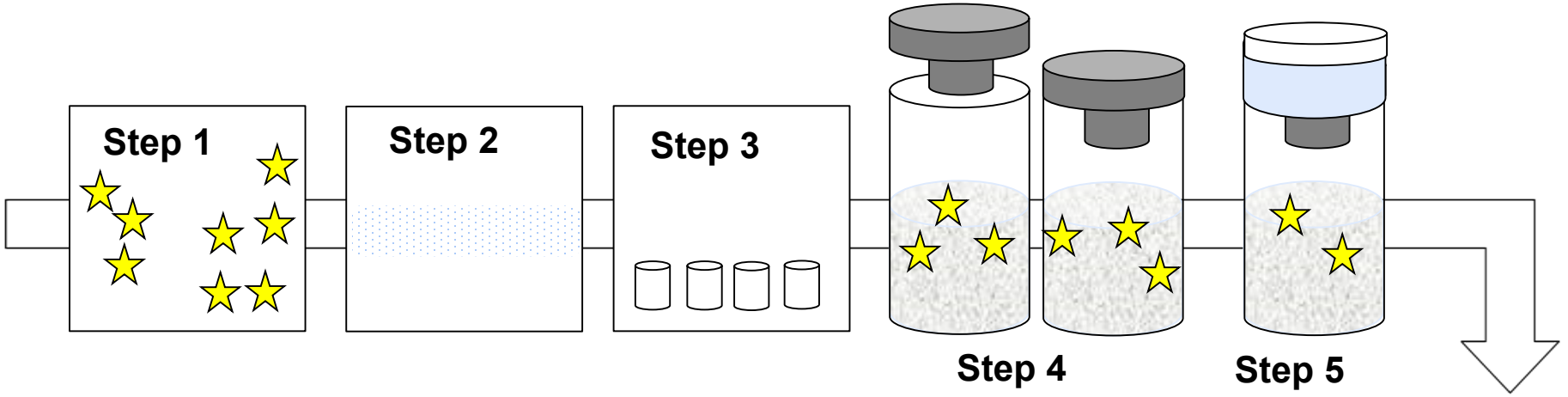
Step 5: Downstream Processing

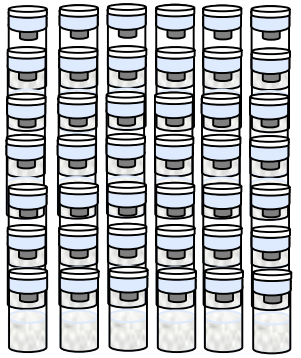


Step 7: Fill & Finish



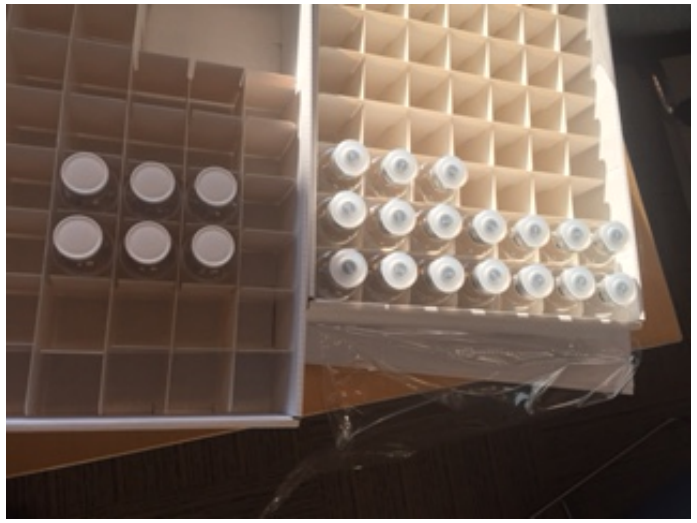
4 bags





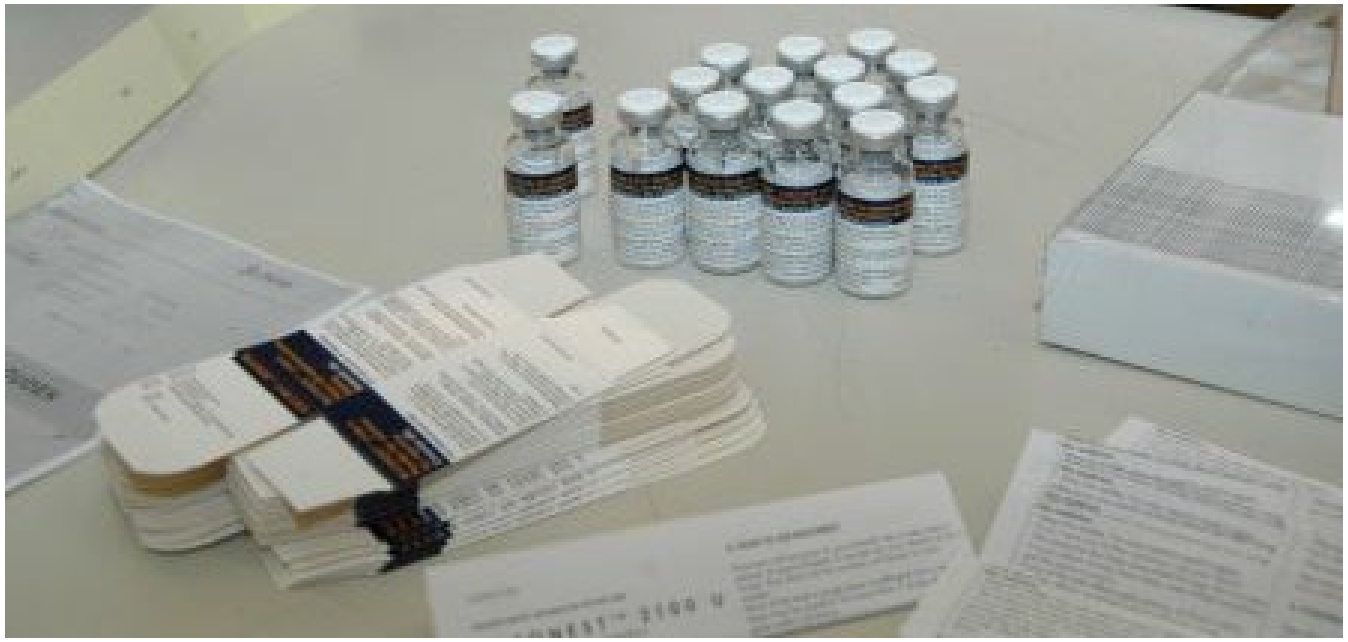
~2500 vials

Step 7: Fill & Finish

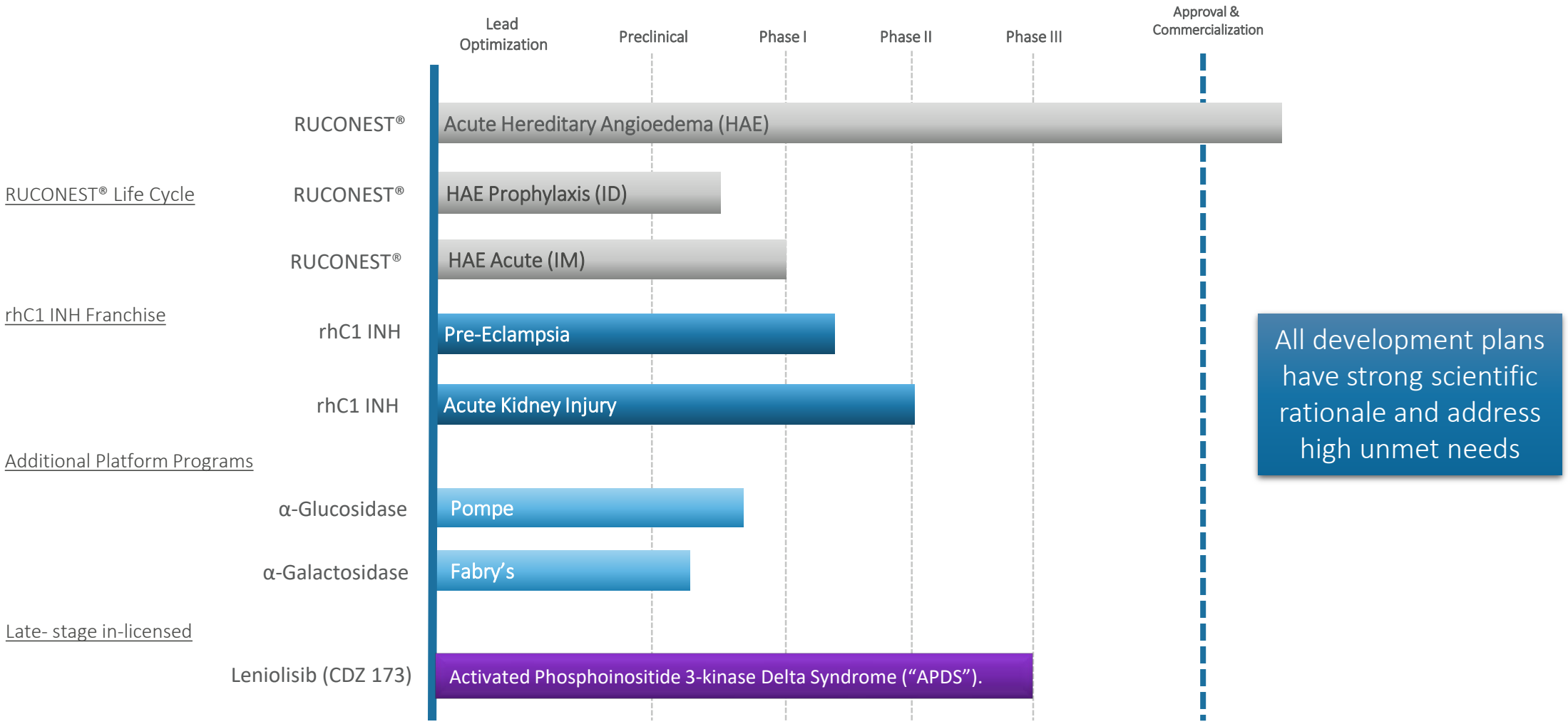


Step 9: Packaging & Labeling

Pharming



High Potential Pipeline



All development plans have strong scientific rationale and address high unmet needs

Acute Kidney Injury (AKI) resulting from Contrast Medium (CM)

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- First described in the 1950's
- Radiographic contrast medium are responsible for 11% of cases of hospital-acquired renal insufficiency, the third most common cause of renal failure after impaired renal perfusion and the use of nephrotoxic medications.
- AKI from CM is responsible for a third of all hospital-acquired acute kidney injury (AKI) and affects between 1% and 2% of the general population and up to 50% of high-risk subgroups following coronary angiography (CA) or percutaneous coronary intervention (PCI).[1]

Table 1 | Risk factors for the development of CIN

Fixed (non-modifiable) risk factors	Modifiable risk factors
Older age	Volume of CM
Diabetes mellitus	Hypotension
Pre-existing renal failure	Anemia and blood loss
Advanced CHF	Dehydration
Low LVEF	Low serum albumin level (<35 g/l)
Acute myocardial infarction	ACE inhibitors
Cardiogenic shock	Diuretics
Renal transplant	Non-steroidal anti-inflammatory drugs
	Nephrotoxic antibiotics
	IABP

Abbreviations: ACE, angiotensin-converting enzyme; CHF, congestive heart failure; CIN, contrast-induced nephropathy; CM, contrast media; IABP, intra-aortic balloon pump; LVEF, left ventricular ejection fraction.

[1] *Mehran R, Nikolsky E. Contrast-induced nephropathy: definition, epidemiology, and patients at risk. Kidney Int Suppl 2006:S11–15*
CIN= Contrast induced nephropathy

Acute Kidney Injury (AKI) Resulting from Contrast Medium (CM)

Pharming

- Screening
- Informed consent
- Randomization
- Baseline samples
- Hydration

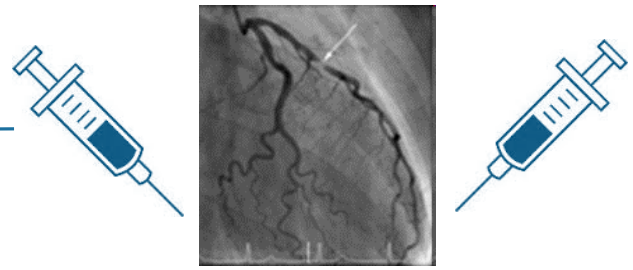
- Blood/urine sampling
- Discharge

Telephone interview

Day -1/0 | Day 0 | Day +1 | Day +2 | Week +4 and +12

Group 1: rhC1INH
<84kg: 50 U/kg
>84kg: 4200 U

Group 2: placebo
NaCl 0.9%

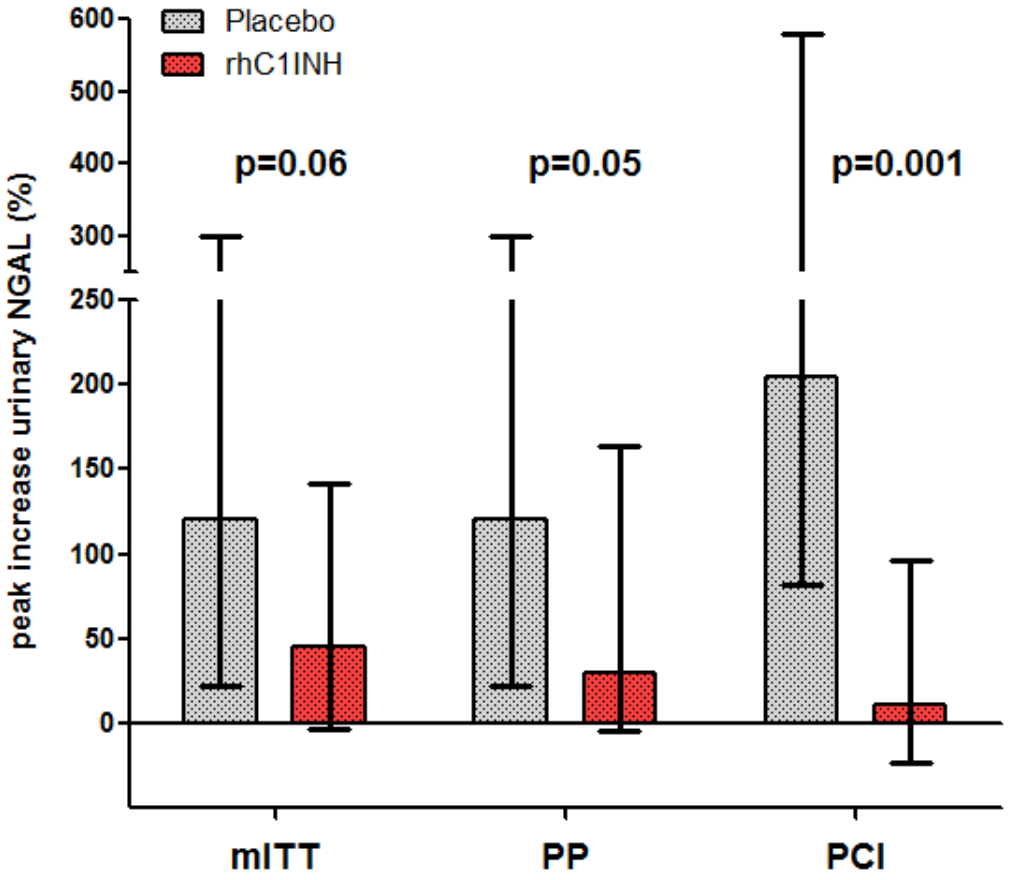


Outpatient visit
- Blood/urine sampling

Study completed M. Osthoff MD et al.,University Hospital Basel, CH

Proof of Concept

Relative urine NGAL peak increase 48 h, (%)



Pre-Eclampsia

Pre-eclampsia (PE), Prevalence, Complications

Pharming

- Pre-eclampsia (PE) has a prevalence of 1-17% throughout the world. Estimated yearly cases of PE in the US alone: 120.000+.

  (Steegers et al., 2010; Osungbade and Ige, 2011)

- Delivery is presently the only therapy of PE, but this is not an option for early PE (from week 20 of gestation).
- The main goal of symptomatic therapy is to prolong gestation of PE patients as far as possible

Panel 1: Maternal and fetal complications in severe pre-eclampsia

Maternal complications

- Abruptio placentae (1–4%)
- Disseminated coagulopathy/HELLP syndrome (10–20%)
- Pulmonary oedema/aspiration (2–5%)
- Acute renal failure (1–5%)
- Eclampsia (<1%)
- Liver failure or haemorrhage (<1%)
- Stroke (rare)
- Death (rare)
- Long-term cardiovascular morbidity

Neonatal complications

- Preterm delivery (15–67%)
- Fetal growth restriction (10–25%)
- Hypoxia-neurologic injury (<1%)
- Perinatal death (1–2%)
- Long-term cardiovascular morbidity associated with low birthweight (fetal origin of adult disease)

Prof. G. Dekker, presentation, Pharming CMD, New York City, June 21, 2018

Pre-eclampsia

Pharming

Table 1 Analytical data (mean ± 1 SD) in normal pregnancy, preeclampsia and in non-pregnant women

	(A) Normal pregnancy (n = 20)	(B) Mild preeclampsia (n = 17)	(C) Moderate preeclampsia (n = 10)	(D) Non-pregnant women (n = 20)
C1-INH activity (%)	74.3 ± 15.5	64.4 ± 14.0	55.5 ± 15.8	95.1 ± 10.8
C1-INH antigen (%)	68.2 ± 10.4	62.7 ± 13.3	53.1 ± 8.8	86.5 ± 12.2

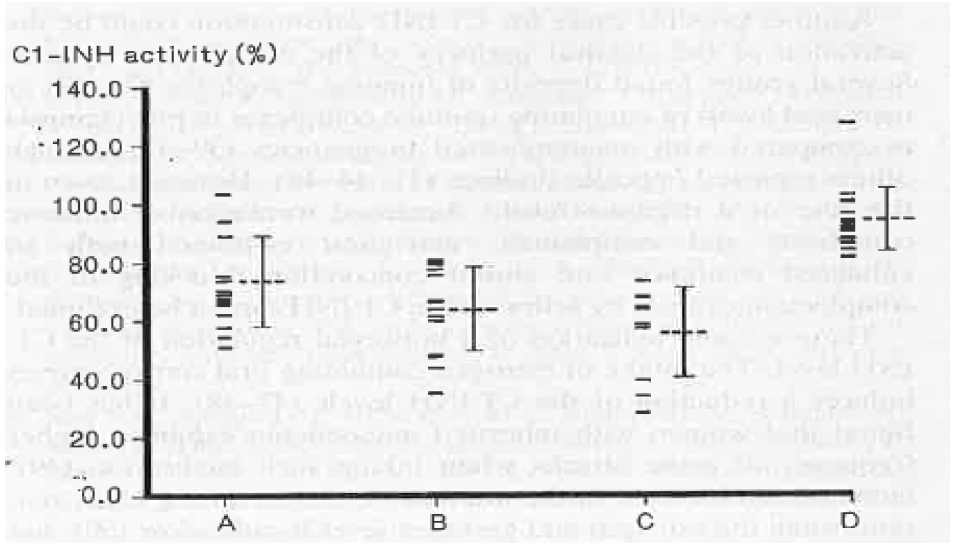


Fig. 2 Scattergram of C1-INH activities. Uncomplicated pregnancies (A), mild preeclampsia (B), moderate preeclampsia (C) and non-pregnant controls (D)

- High unmet need with no current treatment
- Significant cost to healthcare system and families
- Challenging disease to study; demands thoughtful, ethical approach
- Initial clinical study initiated (Netherlands and Australia)

Activated PI3K-δ Syndrome (APDS)

Primary Immunodeficiency and APDS Background

Pharming

- Primary immunodeficiencies (PID) lead to immune system dysregulation with numerous resulting complications
  - Prevalence 1 in 1200
  - More than 300 genes known to cause different PIDs
  - Highly variable clinical presentation, but increased susceptibility to infection is common to most PIDs

- Activated PI3 kinase delta syndrome (APDS) is a PID
  - Caused by autosomal dominant mutations
  - Increased activity of phosphoinositide-3-kinase δ (PI3Kδ)
  - Estimated prevalence 1-2/million
  - More than 240 reported in literature
  - Screening in subset of PID patients has found rates: 5/669 (1%) and 17/184 (9%)
  - Commercially available genetic test

Angulo I, et al. Science. 15;342. 2013. Lucas CL. Nature Immunology. 15, 88-97, 2014.
Michalovich D, et al. Frontiers Immuno. 2018. Jamee M, et al. Clin Rev Allergy Immunology. 2019.

APDS Clinical Spectrum

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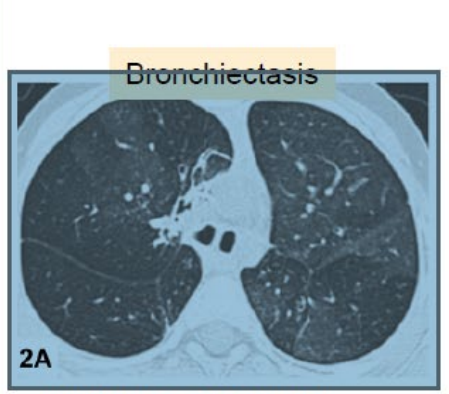
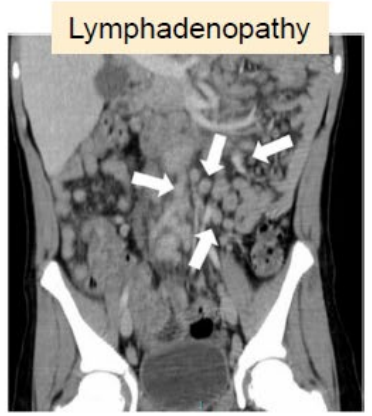
Varying clinical manifestations of symptoms and signs

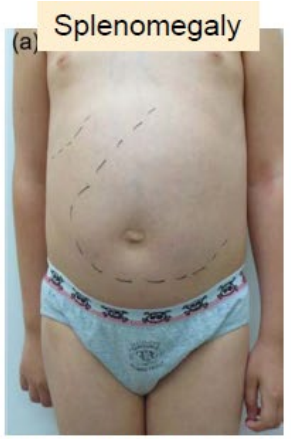
- Recurrent infections
- Organomegaly
- Malignancy
- Autoimmunity



Coulter et al, J.Allerg.Clin. Immunol. 2016



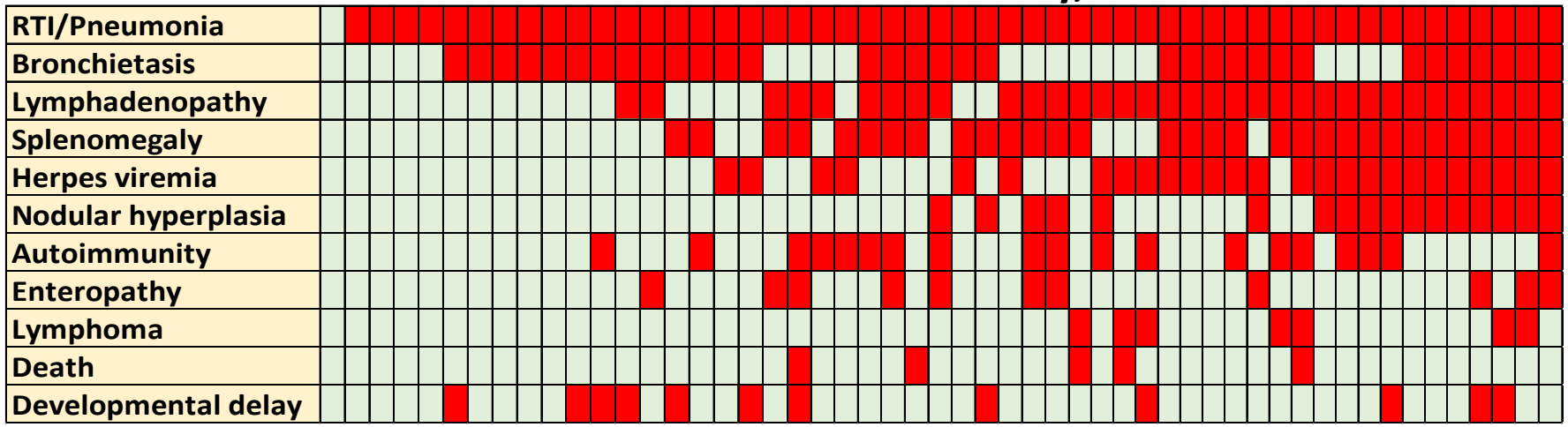
Lucas et al, Nature Immunol, 2014



Elgizouli et al Clin.Exp.Immunol. 2015

Jamee M, et al. Clin Rev Allergy Immunology. 2019.

ADPS Patient Cohort Study, n=53

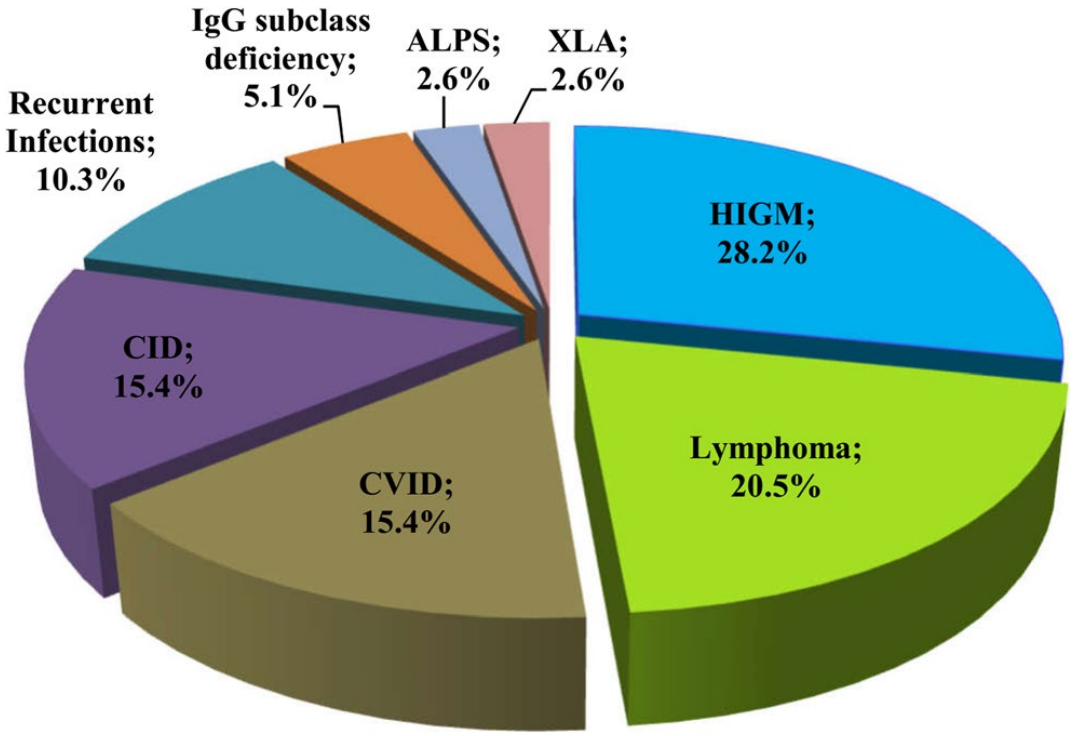


Modified from: Coulter TI, et al. JACI. Vol 139:2, 2017.

= Presence of a complication

APDS Misdiagnosis

Distribution of primary clinical diagnosis of APDS patients



Review of 243 published APDS patients

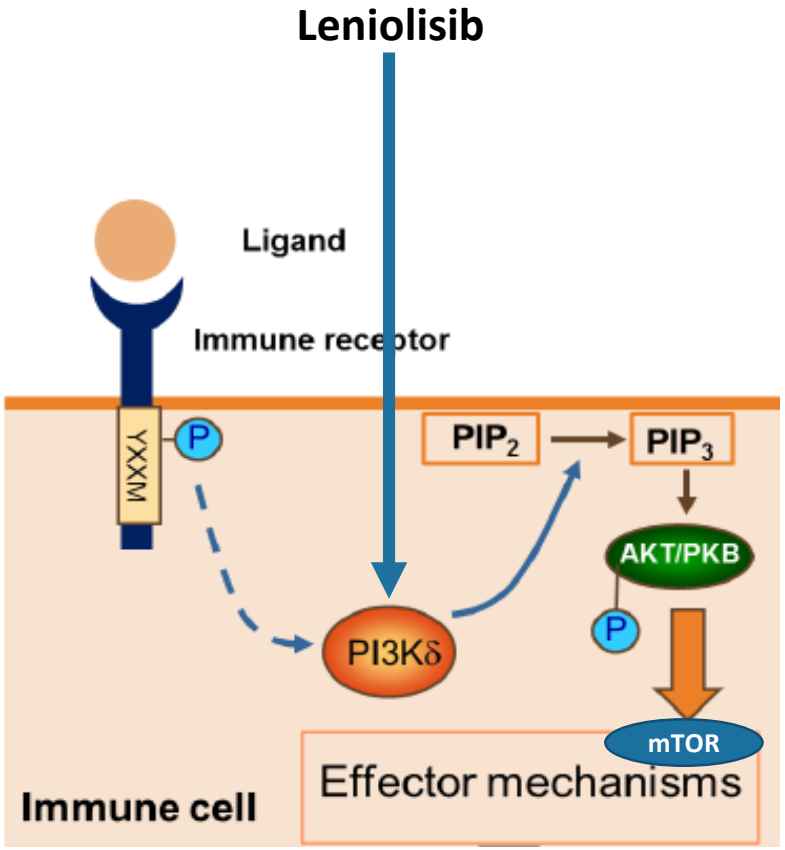
- Symptoms occurred early 1-2 years of age
- Median age diagnosis: 12 years
- Positive family history of PID: 39%

Jamee M, et al. Clin Rev Allergy Immunology. 2019.

APDS Treatment Options

- Current treatment options for APDS:
  - Symptomatic treatment e.g., antibiotics
  - Immune globulin replacement therapy (IVIG/SCIG)
  - Immunosuppressants (e.g. rituximab)
  - Stem cell transplantation
  - Case reports of mTOR inhibitor rapamycin

- Leniolisib
  - Potent, selective PI3Kδ inhibitor
  - Treats the root cause of APDS
  - Orally bioavailable – tablet/capsule
  - Direct PK/PD relationship observed
  - Currently in registration-enabling pivotal study
  - If approved, the drug is expected to reach the market in 1H 2022



Financial Performance & 2019 Outlook

Third Quarter 2019: Financial Results

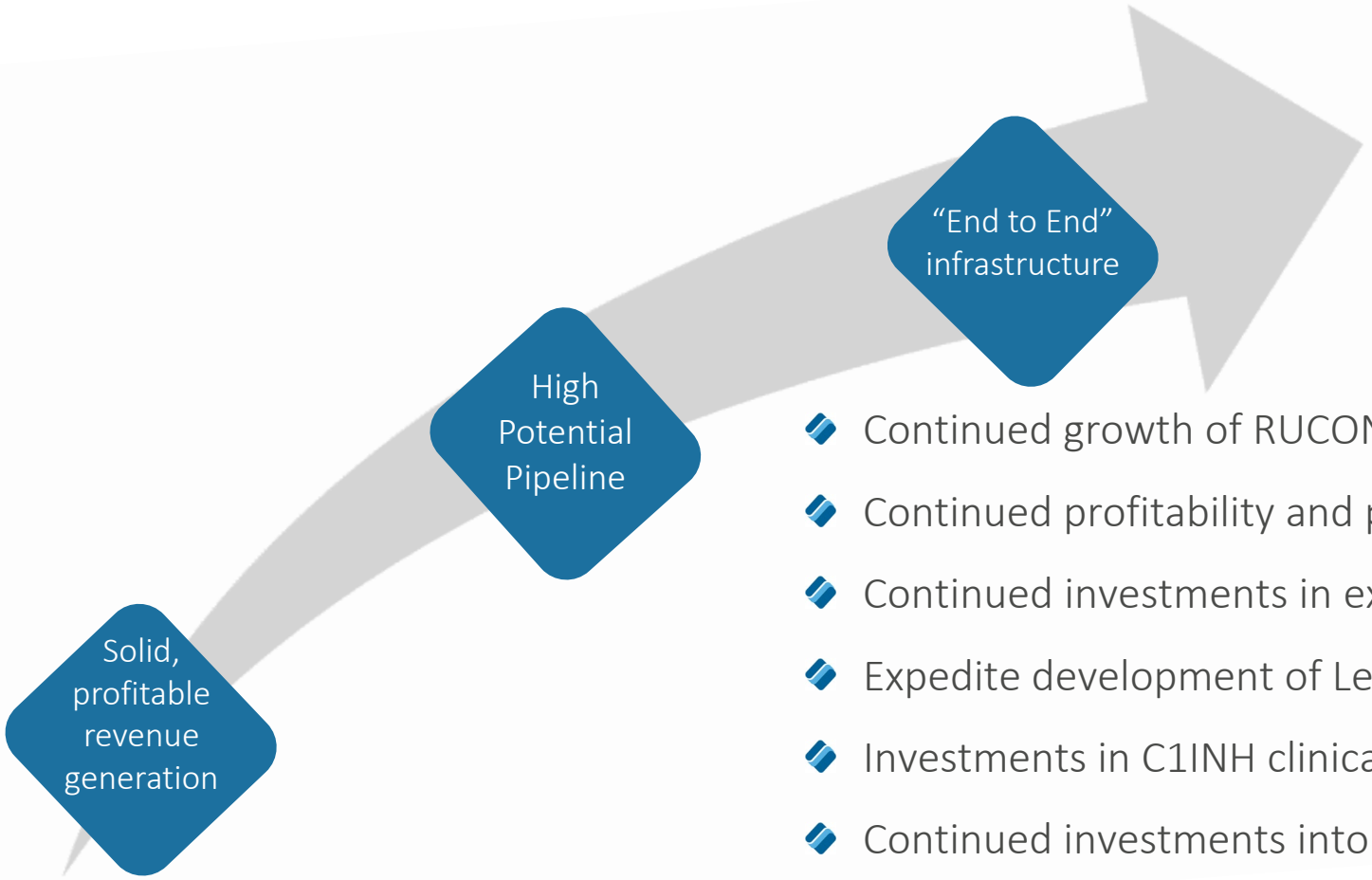
Pharming

9 months to 30 September

Amounts in €m except per share data	*2019 3rd Quarter*	*2019 1st 9 months*	*2018 1st 9 months*	*% Change*
Income Statement				
Revenue from product sales	45.3	122.8	97.7	*26%*
Other revenue	0.2	0.6	0.6	
Total revenue	45.5	123.4	98.3	*26%*
Gross profit	40.1	107.1	82.4	*30%*
Operating result	18.1	42.7	31.0	*38%*
Net result	10.5	24.1	13.9	*73%*
Balance Sheet				
Cash & marketable securities	64.4	64.4	72.2	*(11%)*
Share Information				
Earnings per share (€): - Undiluted	0.017	0.038	0.022	*73%*
- Fully diluted	0.015	0.036	0.021	*71%*

* *After restatement on the basis set out above and in Note 4 to the Financial Statements in the Annual Report 2018.*

Summary and Outlook 2019 and beyond



- Continued growth of RUCONEST® sales
- Continued profitability and positive cash flows
- Continued investments in expansion of manufacturing
- Expedite development of Leniolisib to FDA and EMA approval
- Investments in C1INH clinical trials for PE and AKI
- Continued investments into development PRT for Pompe and Fabry
- Re-evaluation of most advantageous route of administration for RUCONEST®

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www.pharming.com

ENXTAM: PHARM

Bloomberg: PHAR.AS