Pharming Group N.V.

Sijmen de Vries
Chief Executive Officer

Investor Tour Zwolle

9th of January 2020

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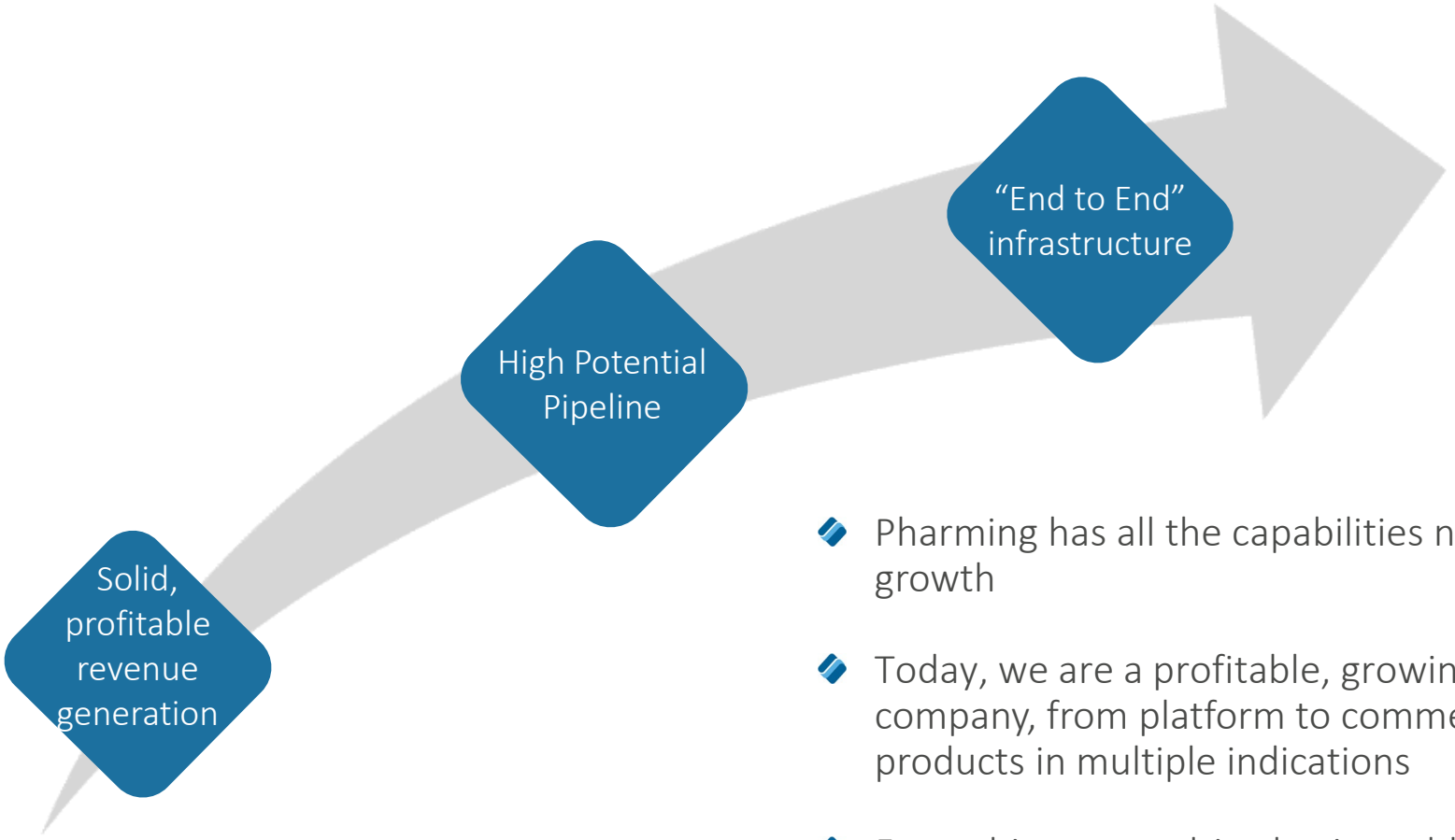
Company Overview

- **Public Company:** Euronext: PHARM: ~€965 million (~$1.07 billion)
- **Located:** the Netherlands, ~220 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

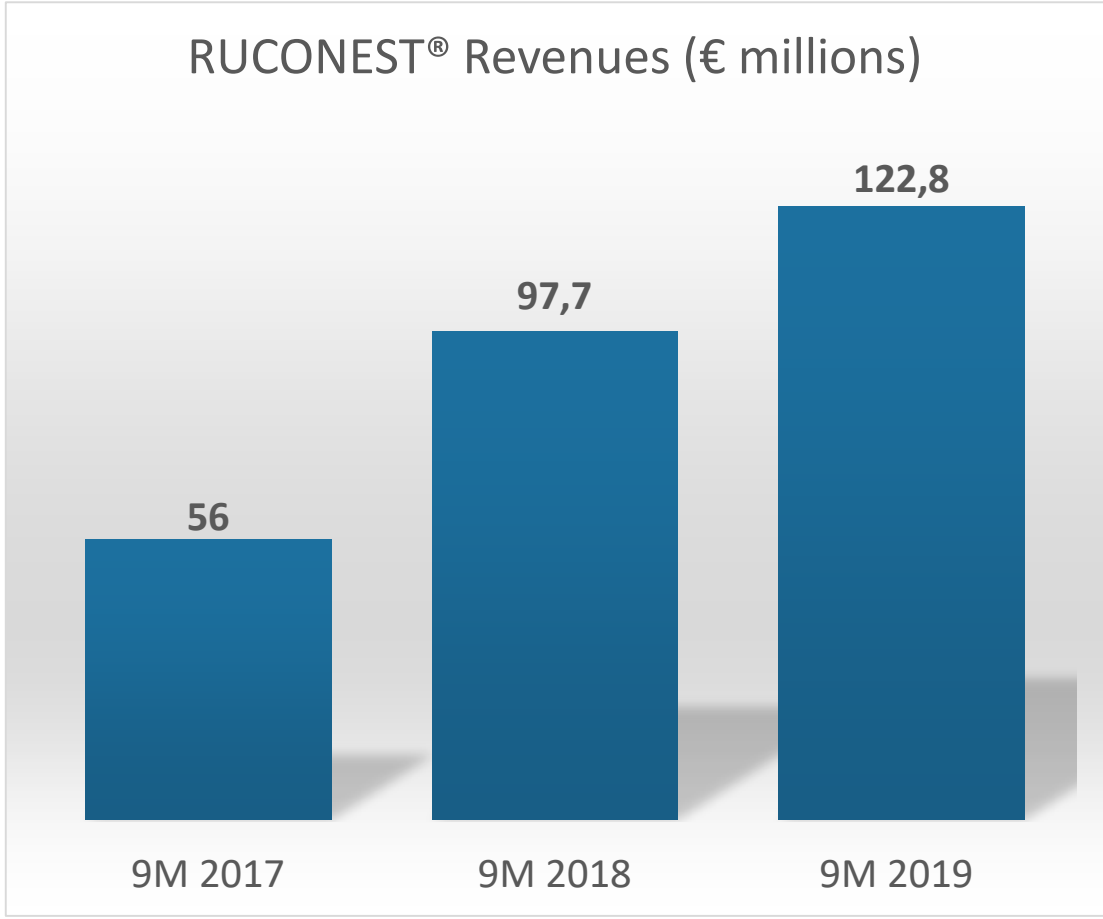


- Pharming has all the capabilities needed for sustainable, high growth
- Today, we are a profitable, growing and fully integrated biopharma company, from platform to commercialization, with multiple products in multiple indications
- From this we can drive lasting additional growth by extending the uses of our existing drug into larger indications and developing innovative solutions in select rare, ultra-rare and specialty diseases

Solid, profitable revenue generation

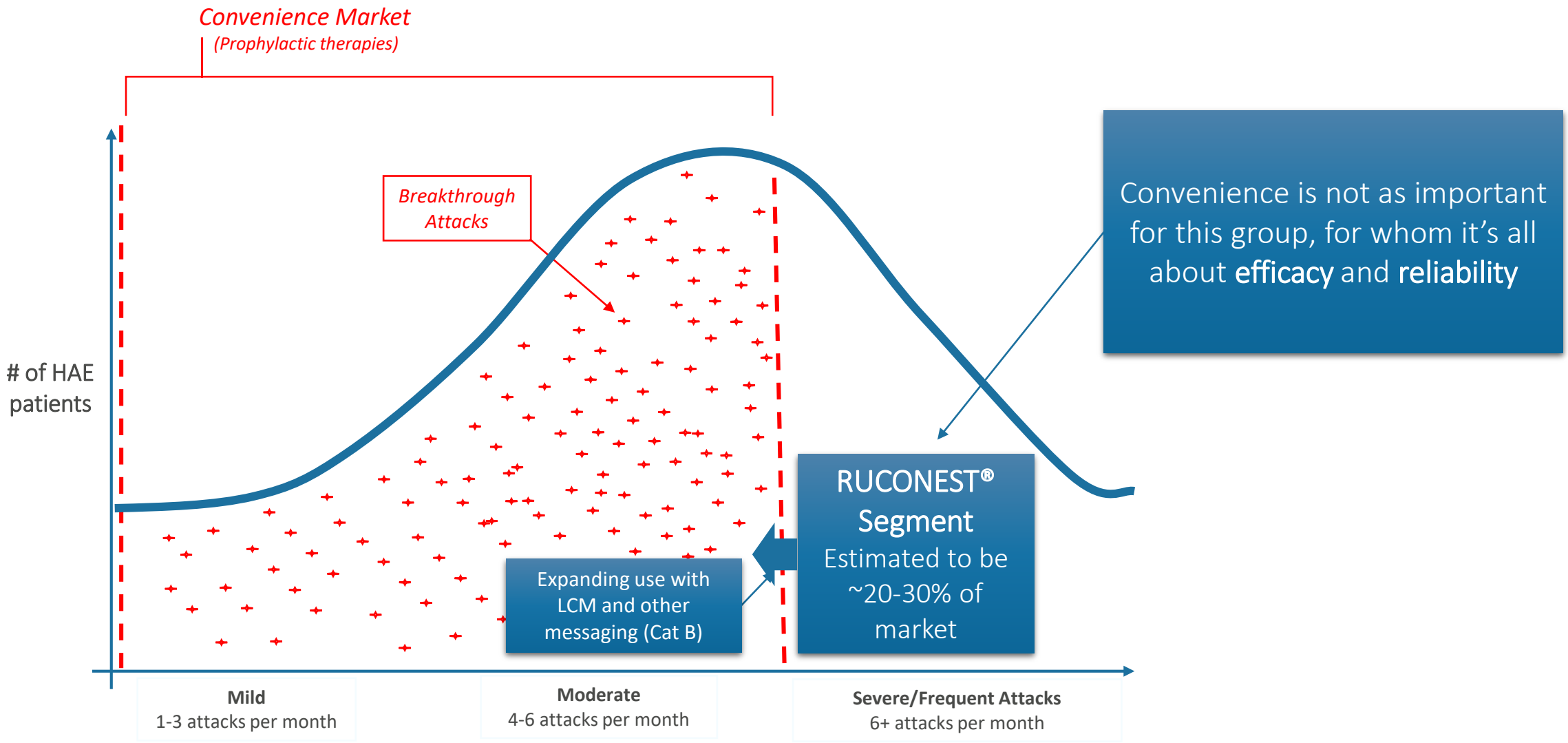
RUCONEST® : Strong Execution of Commercial Strategy

- 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® is the only recombinant PRT, and serves a segment the other therapies are unable to serve in an adequate way, due to its dosing and method of administration
- As a result of the solid RUCONEST® business, Pharming has a strong balance sheet with growing cash position
- New re-acquisition of territories licensed to Sobi in December 2019 allows expansion of EU and RoW sales



Pharming is in a very strong position to continue to grow

RUCONEST®: Patient Segmentation in HAE

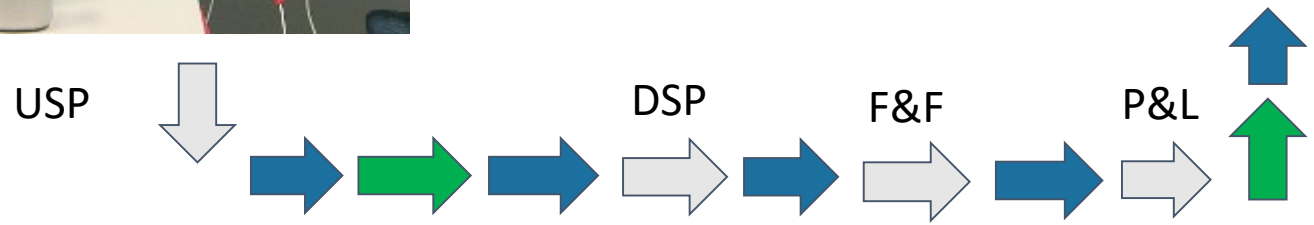


Source: Banerji A, et al. Allergy Asthma Proc. 2018;39:212-223

End to End Infrastructure

From Milk to Medicine

Pharming



Production

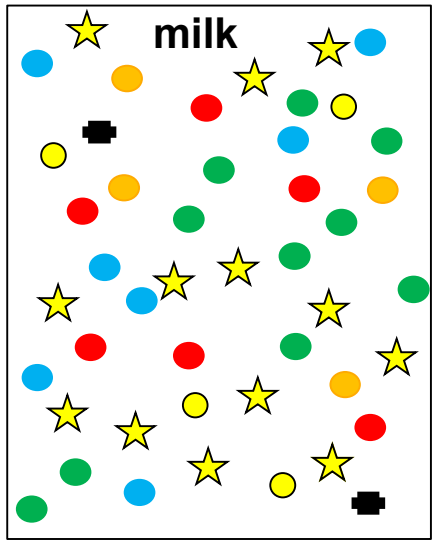
Transport

Storage

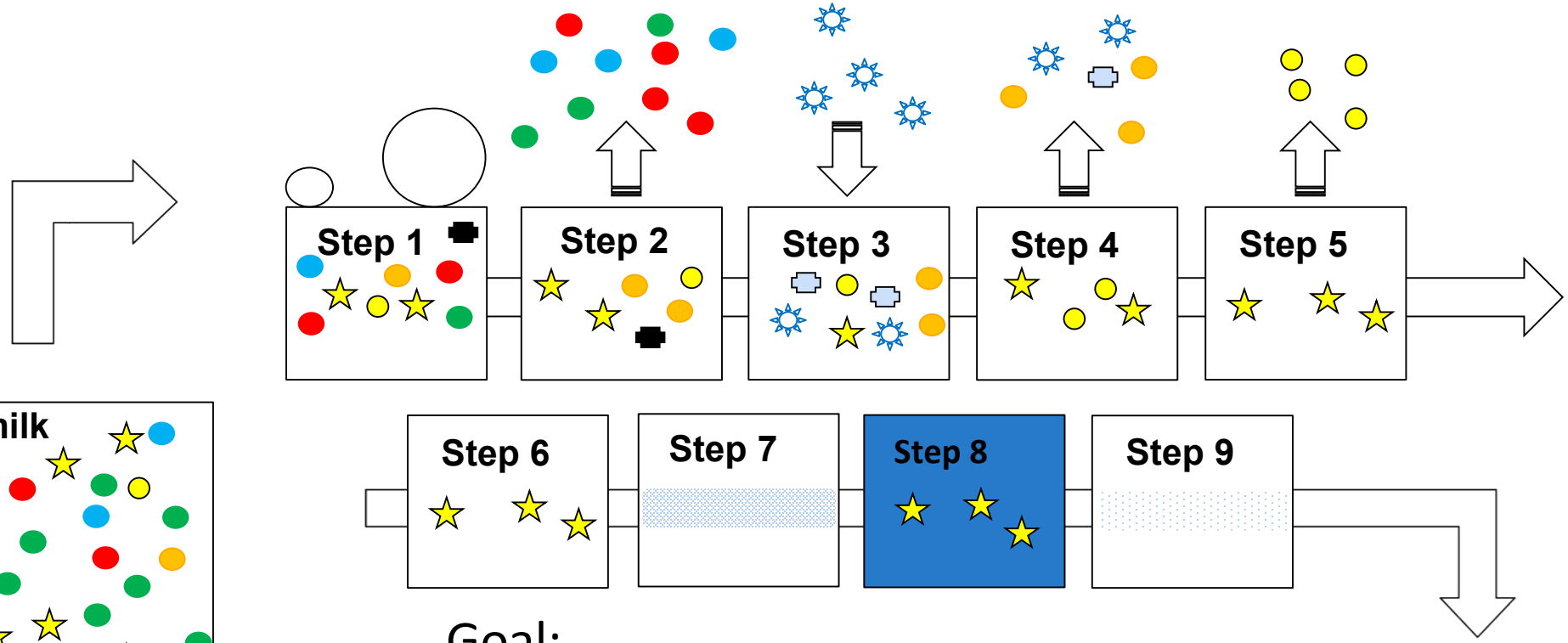
11 steps
Approximately 9 months

Downstream Processing (DSP)

Pharming

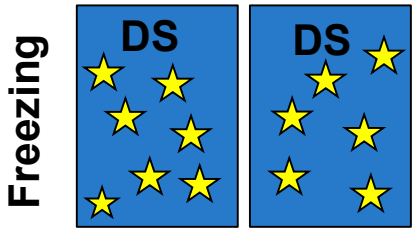


~90 bags



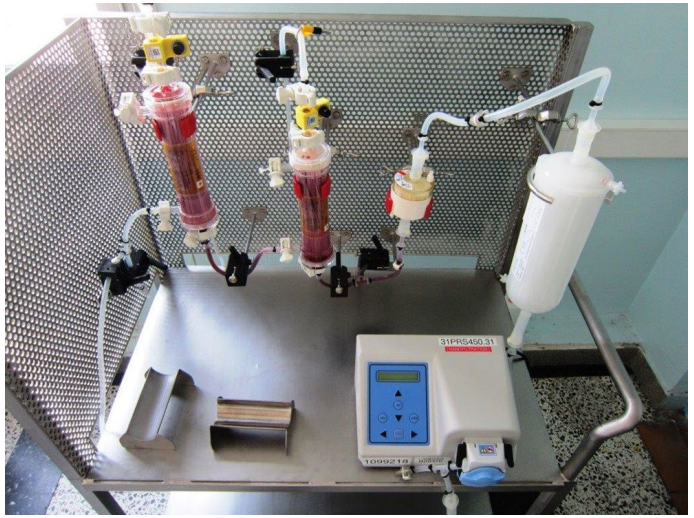
Goal:

- Purification
- Virus inactivation/removal
- Concentration
- Formulation

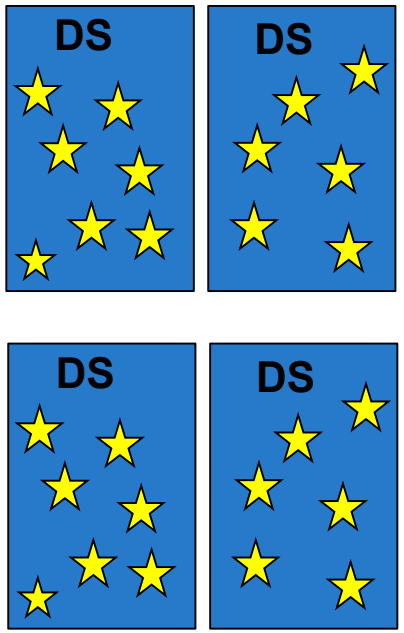


2 bags

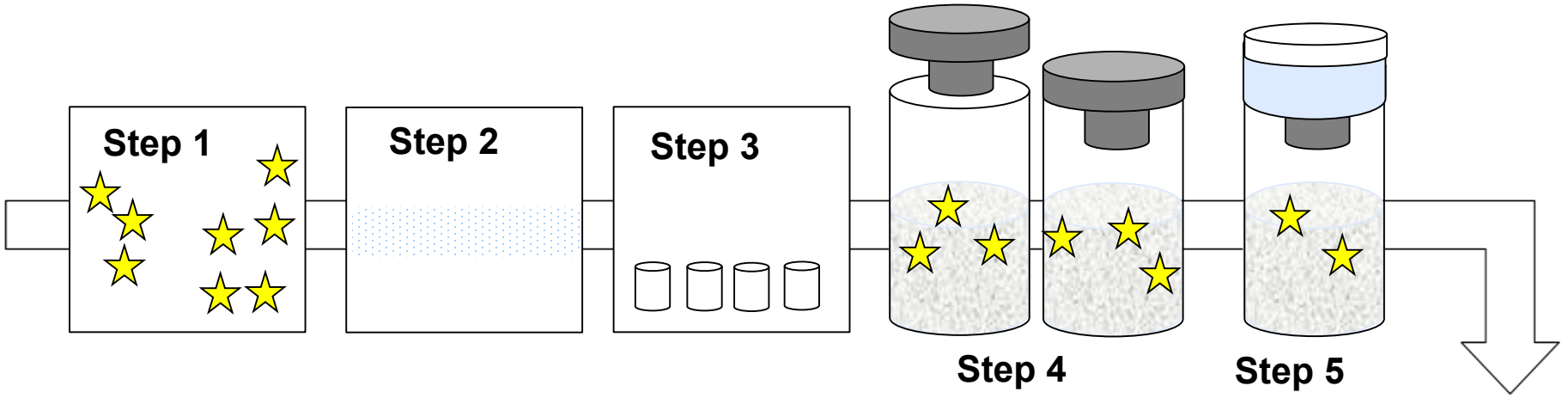
Downstream Processing (DSP)

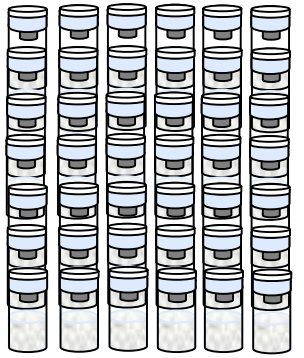


Fill & Finish (F&F)



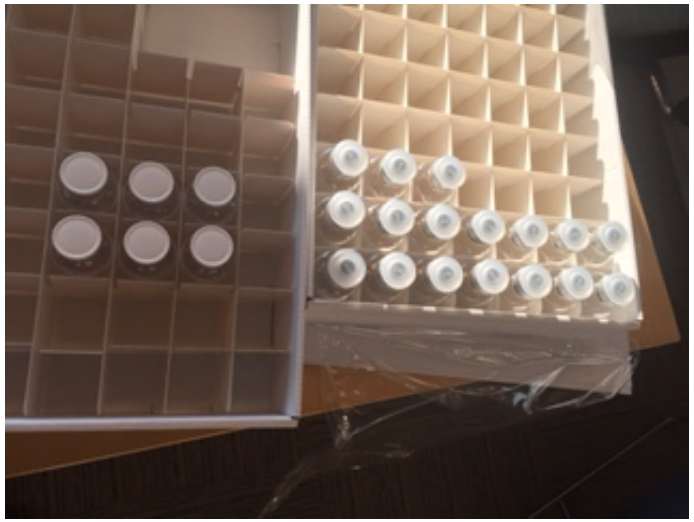
4 bags





~2500 vials

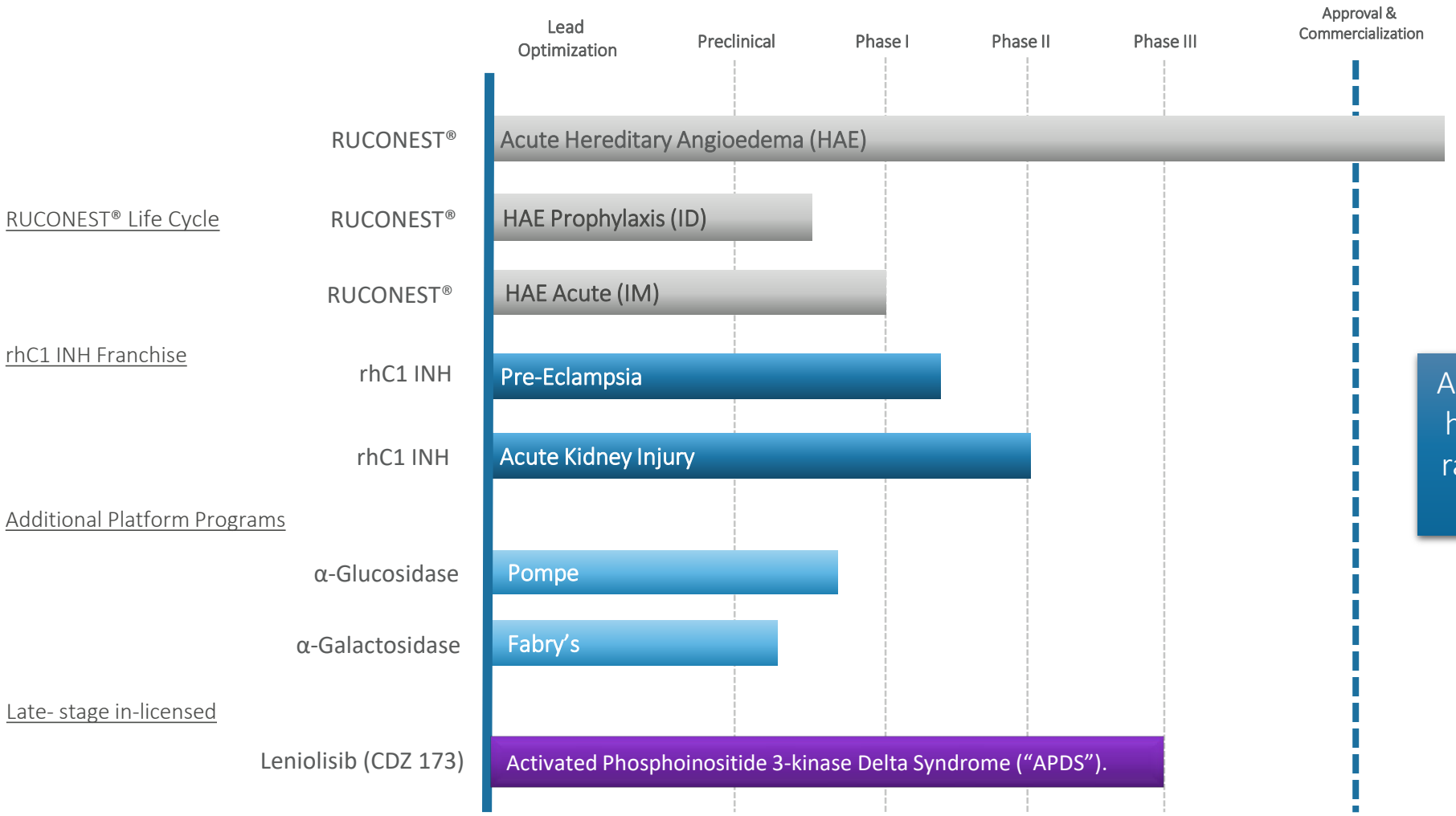
Fill & Finish (F&F)



Packaging & Labeling (P&L)

High Potential Pipeline

High Potential Pipeline



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

Acute Kidney Injury (AKI)

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

- First described in the 1950s
- 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.
- 40 million contrast-enhanced scans per year in the US alone, with around 20% on high-risk subgroup patients
- AKI from contrast media is responsible for a third of all hospital-acquired acute kidney injury (AKI)
- AKI 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]
- No specific therapy available at present: outcomes vary from reversible AKI which requires 7-21 day ICU treatment, through permanent dialysis, renal transplantation and death.

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

Study: AKI Resulting from Contrast Medium (CM)

Pharming

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

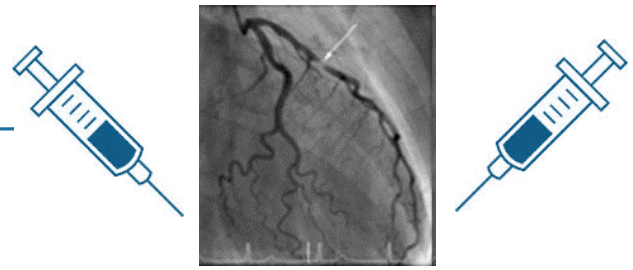
- Blood/urine sampling
- Discharge

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%



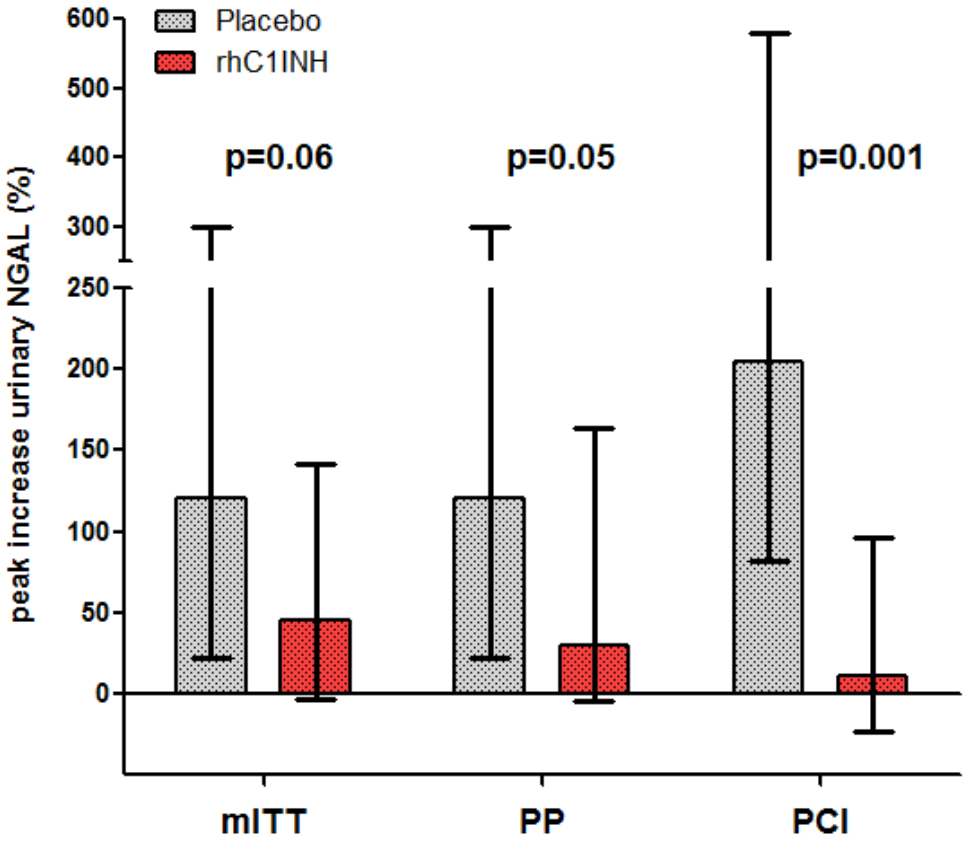
Outpatient visit
- Blood/urine sampling

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

Data - Proof of Concept

- Study of contrast-induced nephropathy at University Hospital Basel reported Oct 2018
- 75 eligible patients given either drug or placebo before and after treatment
- Study used existing HAE dose, and showed clinical and statistical significance
- Results were especially strong in percutaneous coronary intervention patients
- Data has been used to prepare for a study in this specific group led by the same team in Switzerland, starting very shortly

Relative urine NGAL peak increase 48 h, (%)



Pre-Eclampsia (PE)

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

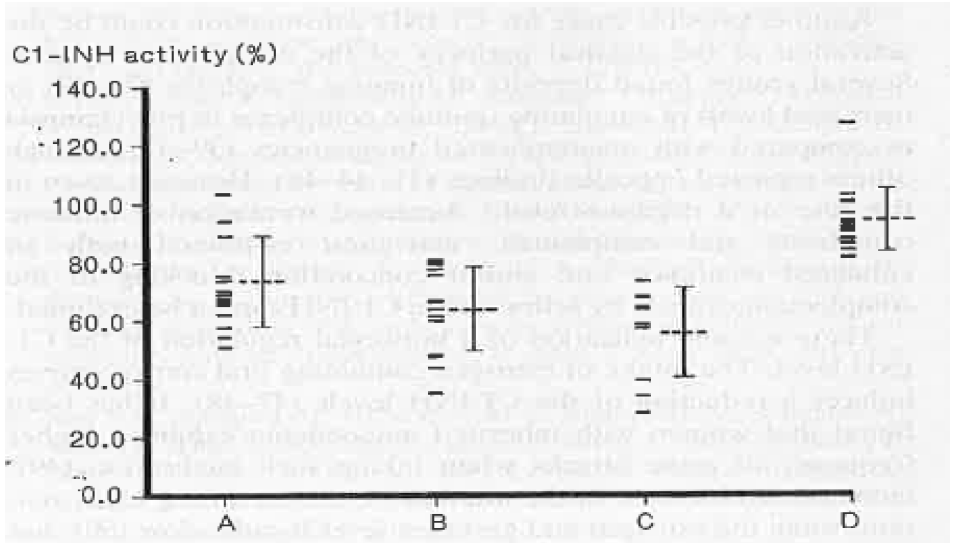


Fig. 2 Scattergram of C1-INH activities. Uncomplicated pregnancies (A), mild preeclampsia (B), moderate preeclampsia (C) and non-pregnant controls (D)

- 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 gene mutations 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 primary immunodeficiency
  - 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

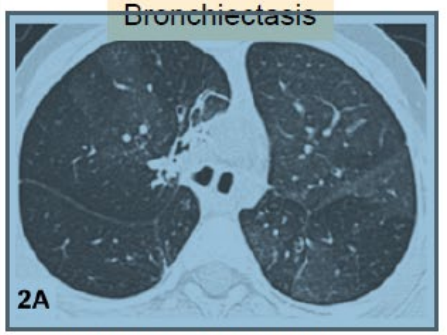
Pharming

Varying clinical manifestations of symptoms and signs

- Recurrent infections
- Organomegaly
- Malignancy
- Autoimmunity

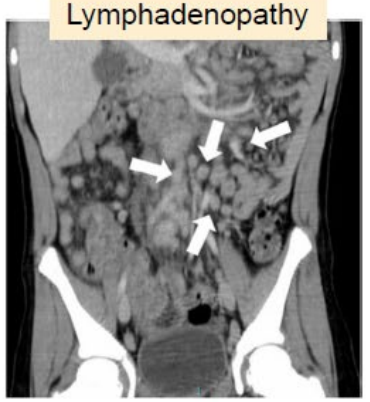
Source: Jamee M, et al. Clin Rev Allergy Immunology. 2019

Bronchiectasis



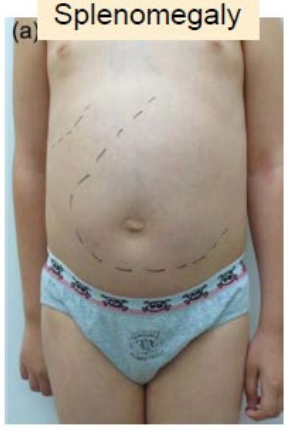
Coulter et al, J.Allerg.Clin. Immunol. 2016

Lymphadenopathy



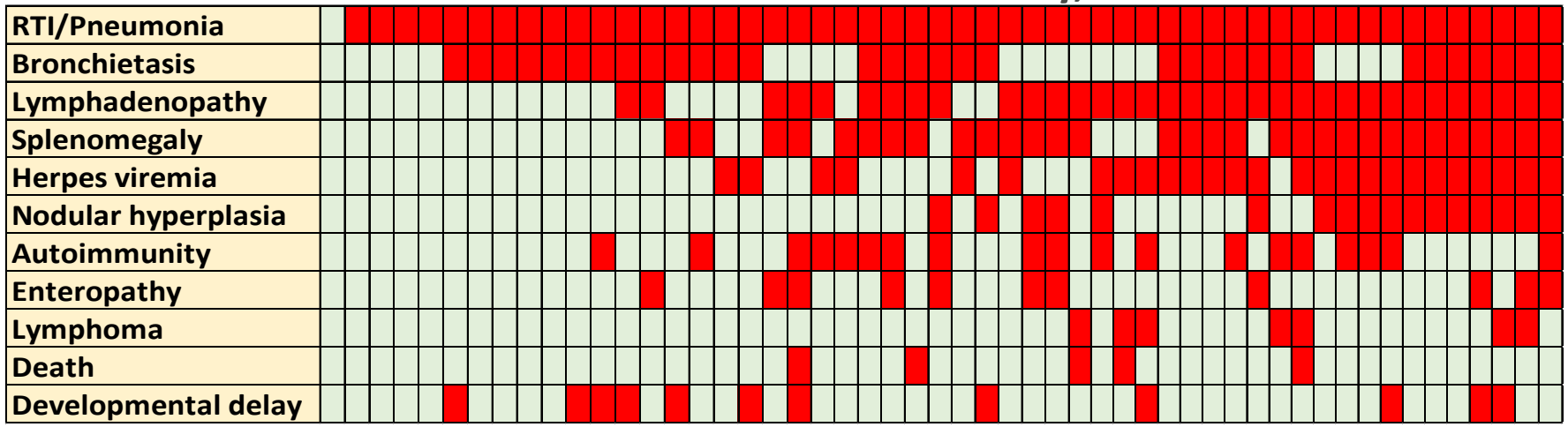
Lucas et al, Nature Immunol, 2014

Splenomegaly



Elgizouli et al Clin.Exp.Immunol. 2015

ADPS Patient Cohort Study, n=53



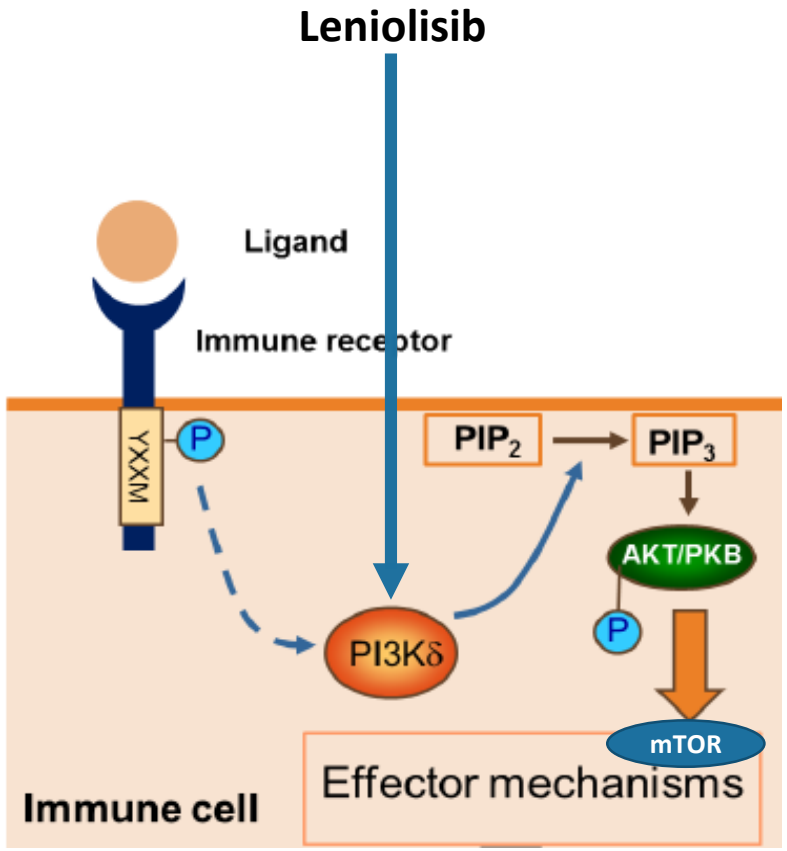
Modified from: Coulter TI, et al. JACI. Vol 139:2, 2017.

= Presence of a complication

APDS Treatment Options

Pharming

- Current treatment options for APDS:
  - Symptomatic treatment e.g., antibiotics
  - Immune globulin replacement therapy (IVIG/SCIG)
  - 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 mid-2022



Financial Performance & 2019 Outlook

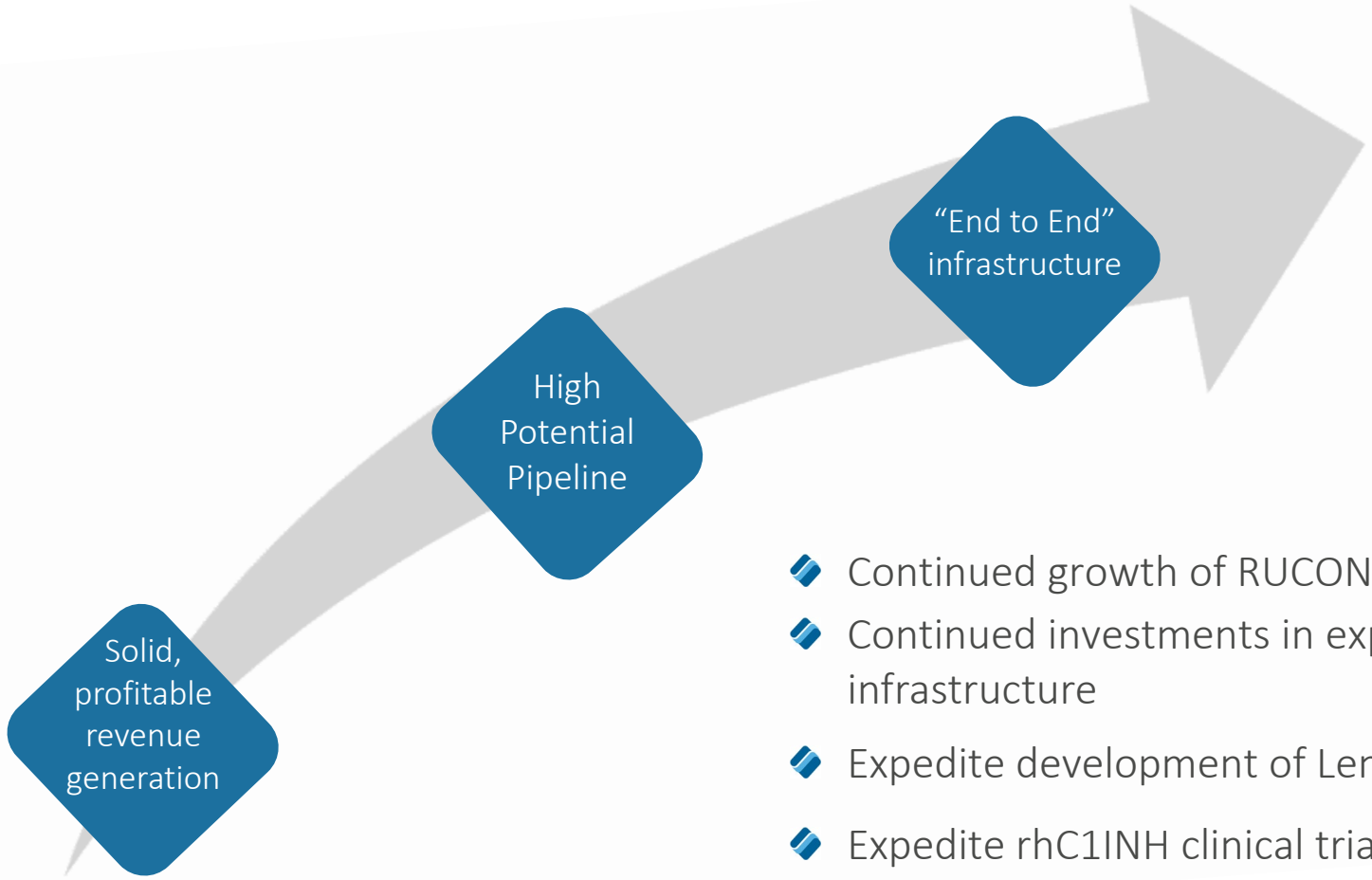
Third Quarter 2019: Financial Results

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%*

Summary and Outlook 2020 and beyond



- Continued growth of RUCONEST® sales, profitability and +ve cashflow
- Continued investments in expansion of commercial & manufacturing infrastructure
- Expedite development of Leniolisib to FDA and EMA approval
- Expedite rhC1INH clinical trials for PE and AKI
- Continued development PRT for Pompe and Fabry
- Re-evaluate most advantageous administration route for RUCONEST®

Pharming

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