Pharming Group N.V.

Sijmen de Vries
Chief Executive Officer

9M 2020 Results Webinar

Amsterdam
The Netherlands
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Forward Looking Statements

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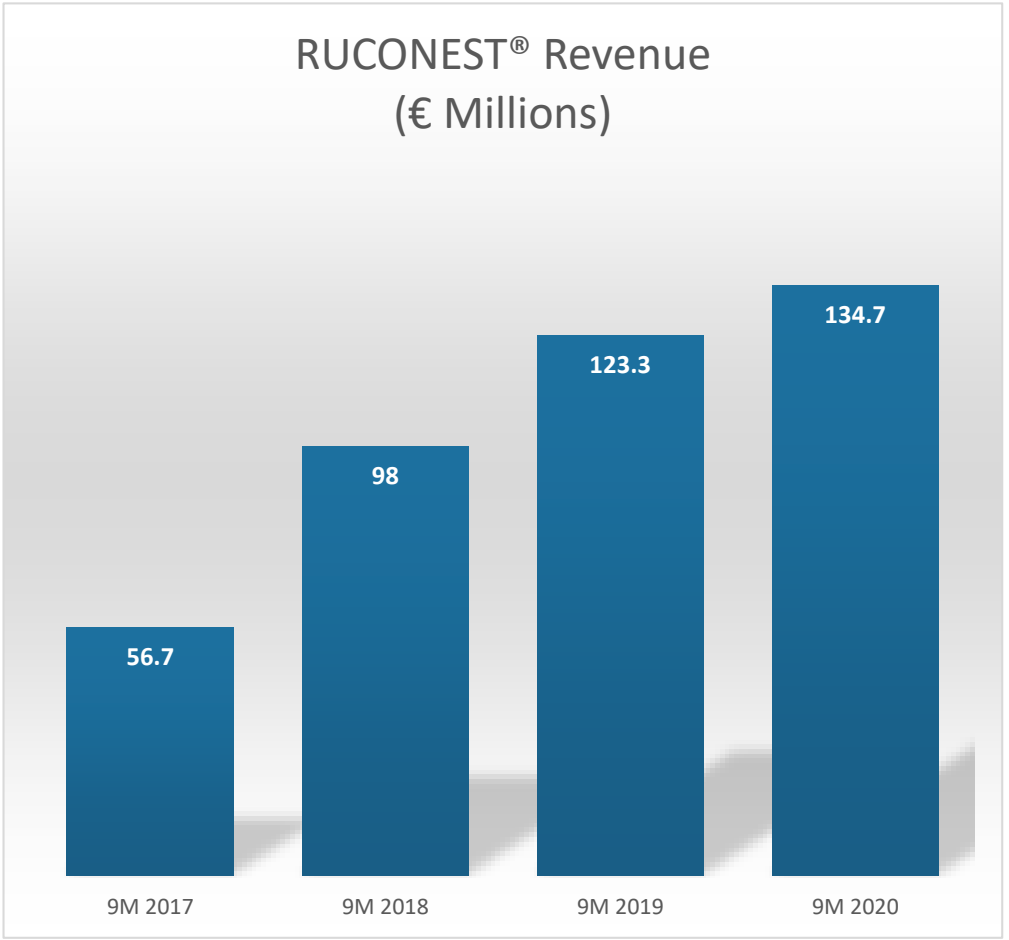
Company Overview

- Established in 1988, based in the Netherlands with 250+ employees
- Listed on the Amsterdam stock exchange: PHARM
- **Rare and ultra-rare disease development and commercialisation, plus large indications for lead product:**
  - Marketed lead product: **RUCONEST® (rhC1INH)**
  - Recombinant human C1-esterase inhibitor (enzyme replacement therapy) developed using our unique technology platform
  - Approved for the treatment of acute angioedema attacks in patients with hereditary angioedema (HAE)
  - Established commercial infrastructure in the USA and EU, and in partnership in Latin America, Korea and Israel
  - Clinical trials for pre-eclampsia, acute kidney injury and COVID-19
- Late-stage in-licenced product: **leniolisib,** for the treatment of Activated Phosphoinositide 3-kinase Delta Syndrome (APDS)

Nine-Month 2020 Results

Financial highlights

- Revenue of €134.7m in 9M 2020, a 9.2% increase year-on-year (9M 2019: €123.4m)
- Operating profit in 9M 2020 increased 19.7% year-on-year to €51.1m (9M 2019: €42.7m)
- Net profit increased 6.2% year-on-year to €25.6m (9M 2019: €24.1m), despite the negative affect of the euro: dollar exchange rates. On a constant currency basis, net profit increased 59% to €34.6m
- Positive cashflows from operations continued during Q3 2020 of €14.1m, resulting in a cash position of €156.0m at 30 September 2020, despite significant negative currency effects on cash reserves and the payment of the final €2.5m milestone to SOBI in Q3 2020
- Other financial expenses totaling €18.3m, includes €4.5m final Orbimed loan payment and €9.0 million negative currency effects on the cash reserves invested in US government securities and continuous inflow of mainly US dollars from revenues



Strong execution of commercial strategy in US, EU & RoW

- **US revenues increased 8% year-on-year to €129.2 million** (9M 2019: €119 million)
  - US quarter-on-quarter revenues strongly recovered, delivering an increase of 17.3% in Q3 2020 (24.7% at constant currency) compared with Q2 2020, demonstrating continued underlying growth in HAE patients using RUCONEST®, following a move to more online sales and marketing activities during the COVID-19 pandemic.
- **EU & RoW revenues increased 26% year-on-year to €5.4 million** (9M2019: €4.3 million)
  - This follows the reacquisition of commercial product rights in EU territories, effective from 1 January 2020, as the Company continues to build out its EU commercial infrastructure and expand into new territories
- To date, the global COVID-19 pandemic has not impacted the availability/distribution of RUCONEST® to HAE patients or the upscaling/continued production of RUCONEST®

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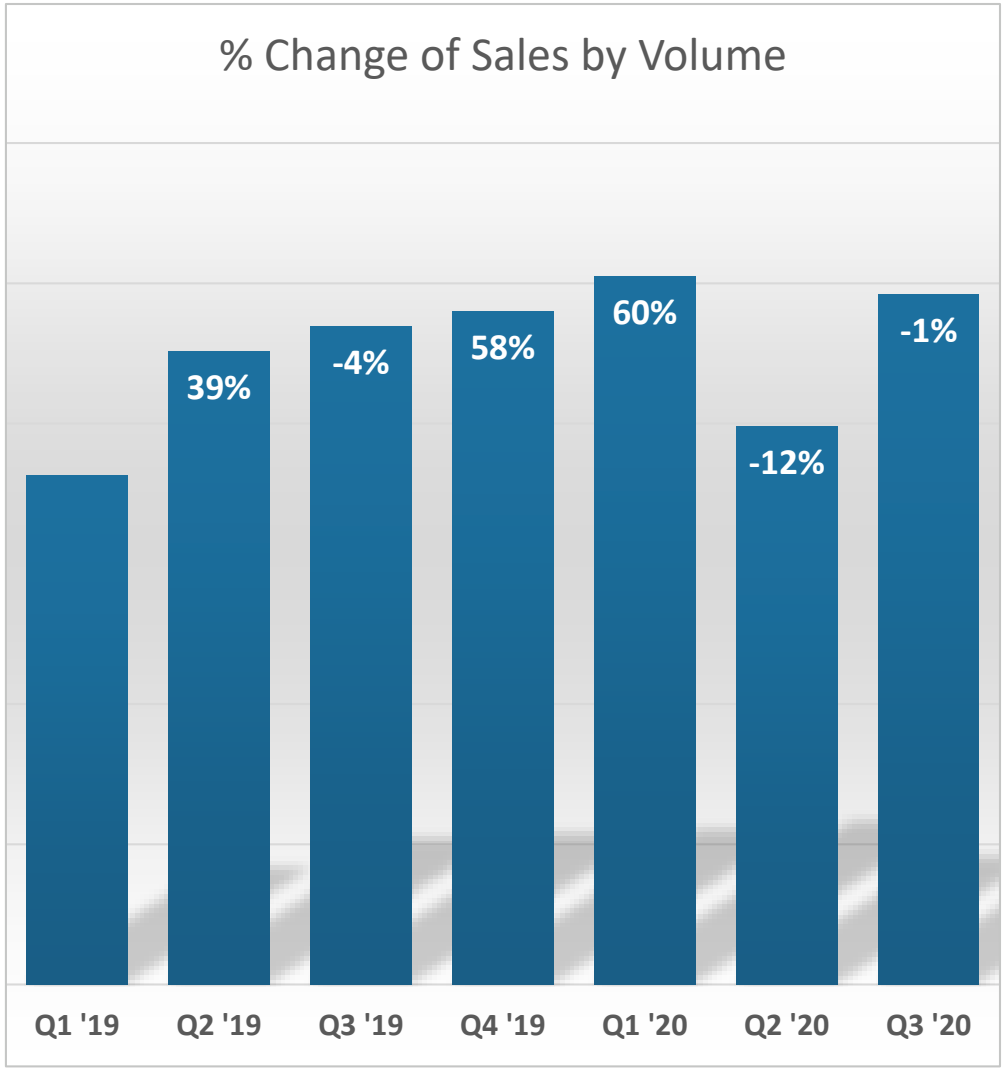


Operational highlights: Q3 2020

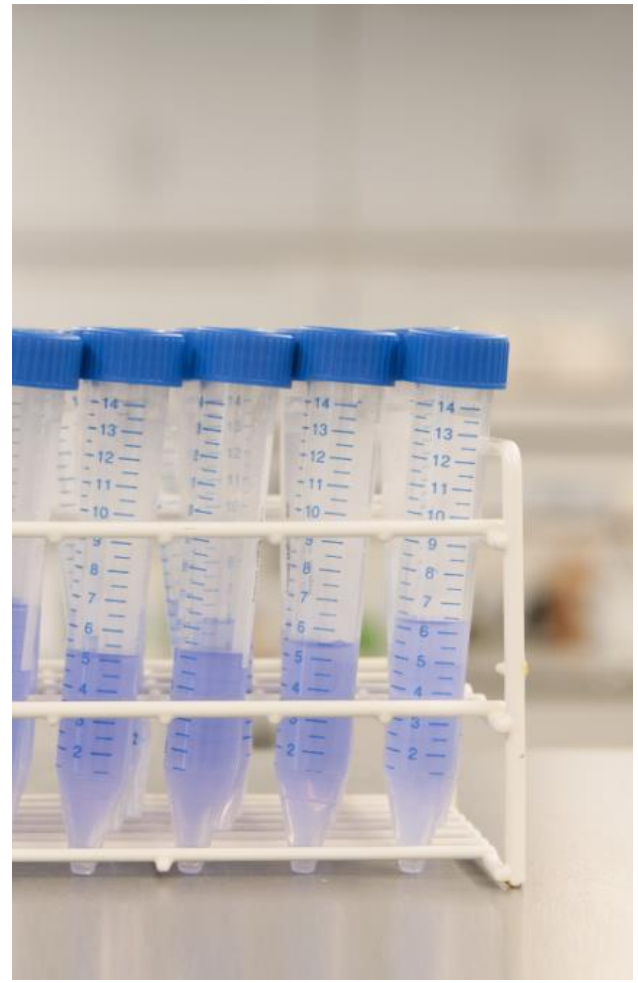
- The first patient was treated in our randomised, controlled, investigator-initiated clinical trial in up to 150 patients for the treatment with rhC1INH of patients with confirmed COVID-19 infections hospitalised with related severe pneumonia at the University Hospital Basel in Basel, Switzerland.
- The publication of data in the peer-reviewed journal, Frontiers in Immunology, from a *compassionate use* programme of five patients with confirmed COVID-19 infections hospitalised with related severe pneumonia that were treated with rhC1INH at the University Hospital Basel in Basel, Switzerland.
- In October, the European Commission has granted orphan drug designation for leniolisib for the treatment of APDS. The orphan drug designation provides certain regulatory procedural and financial incentives including, but not limited to, product market exclusivity for ten years in the EU following regulatory approval.
    - Leniolisib was previously granted Orphan Drug Designation by the US Food and Drug Administration (FDA) in January 2018.
- Initiated process for secondary listing in the US on Nasdaq via a Level 2 ADR programme, which **does not include plans for fundraising** due to the strong financial position of the Company
    - EGM convened for 11 December 2020 for a proposal to amend the Company's Articles of Association to implement a one-tier Board structure in anticipation of the launch of an ADR Programme and the associated Nasdaq listing

Impact of COVID-19 on Pharming's business

Pharming continues to comply with international guidance and requirements across its operations to prioritise the health and safety of its employees during the COVID-19 pandemic.

An update on the impact of COVID-19 on the operations of the business is summarised below:

- No impact on the upscaling or continued production of RUCONEST®
- No impact on the availability or distribution of RUCONEST® to HAE patients
- The recruitment of new patients in ongoing clinical trials has been temporarily halted; patients already incorporated into ongoing clinical trials are continuing to receive treatment
- As a result of halted recruitment, timelines for the pre-eclampsia and acute kidney injury studies are expected to incur delays, subject to the return of recruitment
- Recruitment in the registration enabling trial for leniolisib has restarted and subject to regulatory approval, we continue to expect the potential launch of leniolisib in H2 2022



Investing for long-term growth

Three-pillar strategy for growth

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Continuing to grow RUCONEST® sales through further country launches & increasing HAE market share

- Fully commercialise RUCONEST® in all major international markets with our own sales forces
- Improve convenience of therapy for HAE patients
- Evaluate new technologies to treat HAE

Grow our HAE franchise

Expanding indications for rhC1INH & developing new recombinant proteins using our platform technology

- Developing rhC1INH for additional large unmet indications
- Leverage our transgenic manufacturing technology to develop next-generation protein replacement therapies

Extend rhC1INH franchise to larger indications and develop new Enzyme Replacement Therapies

In-licensing or acquiring late-stage clinical development candidates

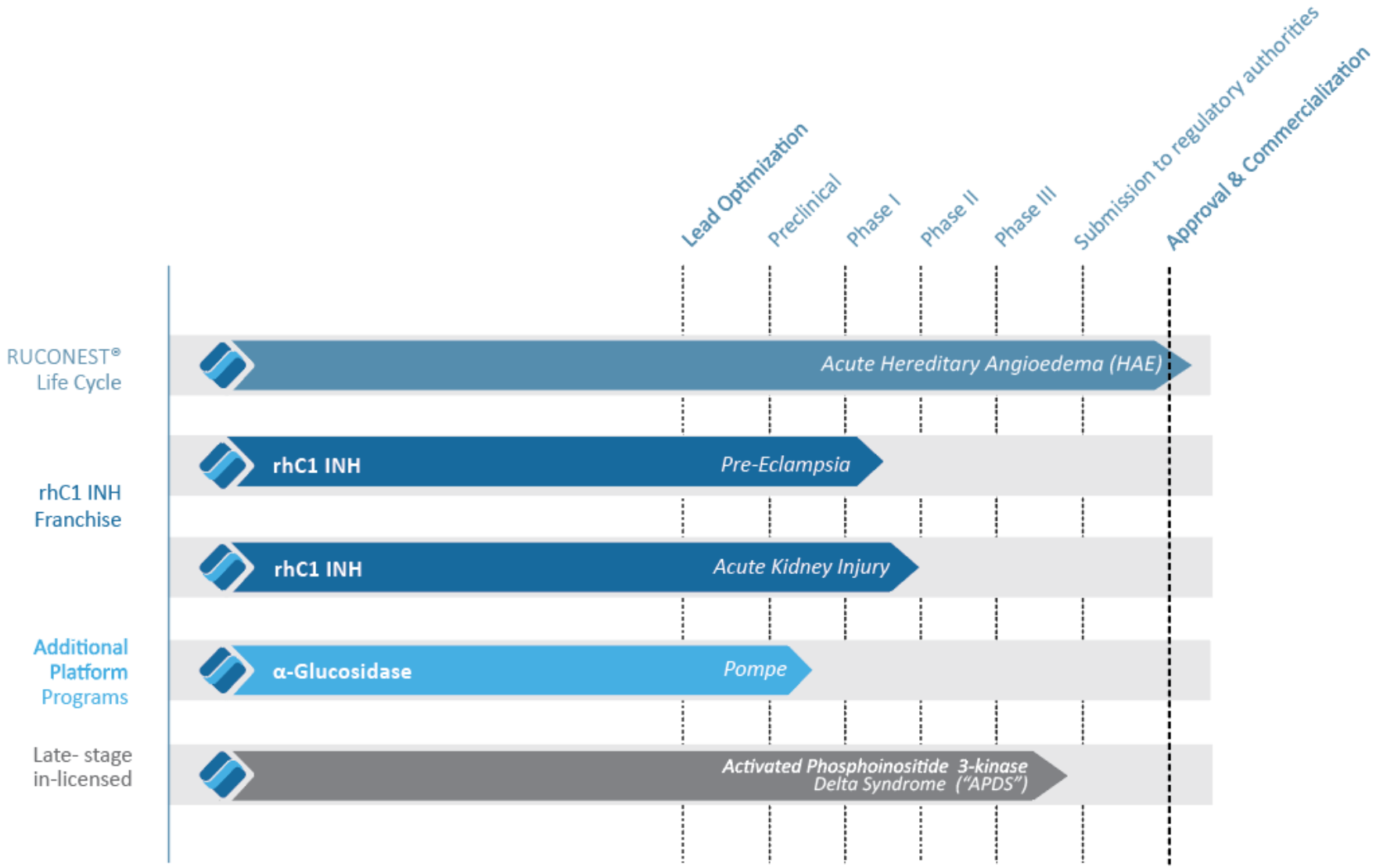
- Developing leniolisib for the treatment of ADPS
- Developing or acquiring new programs or companies that can be commercialized using our sales and marketing infrastructure

Leverage commercial infrastructures and accelerate expansion of portfolio

Investment to increase capacity due to strong demand

- Underlying demand for RUCONEST® increasing further in both US and RoW
  - At the beginning of 2020, Pharming received both European Medicines Agency (EMA) and US Food and Drug Administration (FDA) approval of its new production facility of starting material for RUCONEST®
  - Work initiated on a third facility to safeguard future growth in HAE supplies
  - Plans for a larger fourth facility to manufacture our other pipeline products
  - Strategic investment supports capacity expansion that will help Pharming to meet growing demand for RUCONEST® and long-term expansion of pipeline
- Patient numbers in new indications in pre-eclampsia, acute kidney injury and severe pneumonia as a result of COVID-19 infection are much larger than for HAE
- Re-developing rhC1INH from cattle as a new variant to meet future demand for these such large indications
- In addition, building our own downstream processing facility (to purify the drug from the milk) will enable us to perfect in-house process before anticipated positive clinical data in new indications
- Capacity will only be built on a conservative as-needed basis, bearing in mind lead times, cost and scale involved
- Funding will come from current cash generation

Main Pipeline Projects



Leniolisib – a late-stage product for APDS

- Activated PI3 kinase delta syndrome (APDS) is a primary immunodeficiency (PID)
  - Caused by autosomal dominant mutations
  - Increased activity of phosphoinositide-3-kinase δ (PI3Kδ) leads to malfunctioning B-(immune) cells, symptoms include; recurrent respiratory infections, organomegaly, malignancies and auto-immunity
  - Estimated prevalence 1-2 patients per 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
- Current treatment options for APDS
  - Symptomatic treatment e.g., antibiotics
  - Immune globulin replacement therapy (IVIG/SCIG)
  - Stem cell transplantation
- 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
  - Recruitment in the registration enabling trial has resumed following temporary halt due to COVID-19 pandemic
  - Expected the launch H2 2022

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.

New opportunities for rhC1INH

- Clinical trial for rhC1INH in pre-eclampsia; the recruitment of new patients is temporarily halted due to COVID-19
- Clinical trial for rhC1INH in acute kidney injury in patients undergoing percutaneous coronary interventions such as stent insertions and valve replacements; the recruitment of new patients is temporarily halted due to COVID-19
- Clinical trials for rhC1INH in COVID-19;

The University Hospital of Basel, Basel, Switzerland: Investigator-initiated clinical trial.

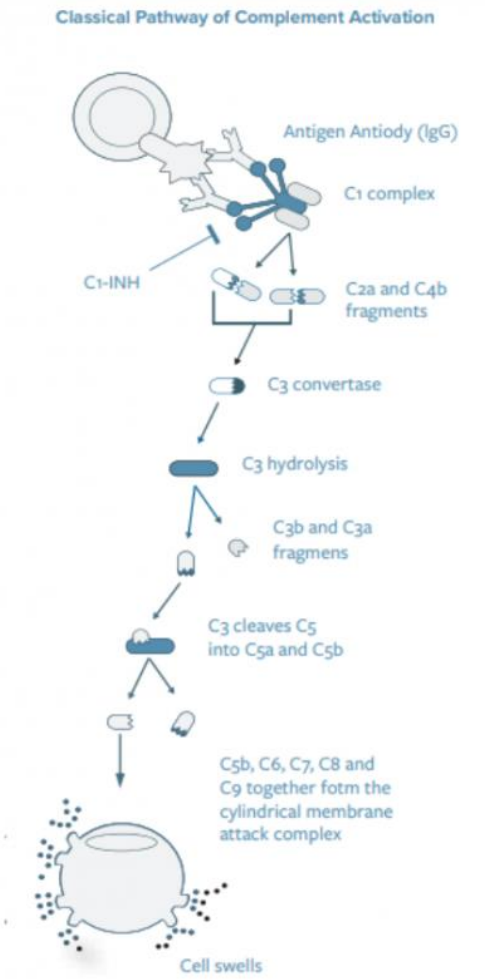
- A phase 2 multinational, randomized, controlled investigator-initiated study of up to 150 patients led by Prof. Michael Osthoff from the University Hospital of Basel continues to recruit.
- This trial has been extended to multiple centres in Switzerland including St. Gallen and Zurich. It is also in the process of being extended to centres in Brazil and Mexico.

Pharming Technologies' clinical trial in the USA: Prevention of Severe SARS-CoV-2 Infection in Hospitalized Patients With COVID-19

- A randomized, open-label, parallel-group, controlled, multi-center trial in the United States.
- A phase 2 trial that will include 120 participants.

rhC1INH and severe complications of COVID-19 infections

- C1INH is a protein that naturally occurs in the human body. It regulates several inflammatory pathways in the body by inhibiting certain proteins that are part of the human immune system.
    - In diseases like HAE, deficiency of functional C1 inhibitor leads to excessive activation of the complement system and other immunological and haemostatic pathways, giving cause to angioedema attacks.
- Systemic hyperinflammation is a hallmark of more severe stages of COVID-19 leading to acute respiratory distress syndrome, mechanical ventilation and ultimately death.
- C1 inhibitor production naturally increases during inflammatory conditions, such as infections. Despite this, a relative deficiency may occur and complement activation continues unchecked, often leading to a cytokine storm.
- Treatment with rhC1INH may;
    - dampen uncontrolled complement activation and collateral lung damage,
    - reduce capillary leakage and subsequent pulmonary edema by direct inhibition of the kallikrein-bradykinin system, and
    - reduce the generation of microthrombi by inhibiting MASP-1 induced clot formation and factor XII amplified thrombo-inflammation.



2020 Outlook

Outlook for 2020

For the remainder of 2020, the Company expects:

- Subject to progression of the COVID-19 pandemic in the US; continued growth in revenues from sales of RUCONEST®, mainly driven by the US and expanded EU operations
- Maintenance of positive net earnings during the year
- Continued investment in the expansion of production of RUCONEST® in order to ensure continuity of supply to the growing markets in the US, Europe, China and the RoW
- Investment in the ongoing clinical trials for pre-eclampsia and acute kidney injury, severe pneumonia as a result of COVID-19 infection and support investigators wishing to explore additional indications for rh C1INH
- Investment in the continuing registration-enabling study for leniolisib for APDS, leading to headline data in mid 2021 and launch in H2 2022
- Investment in upscaling and IND enabling studies for α-glucosidase in Pompe disease
- Investment in acquisitions and in-licensing of other new development opportunities and assets as these occur
- Increasing marketing activity where this can be profit-enhancing for Pharming
- Continued close monitoring of the ongoing COVID-19 pandemic and the potential impact on the business

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Q&A

Q&A section

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Question 1: XXXXXX

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Question 2: XXXXXX

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Question 3: XXXXXX

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Question 4: XXXXXX

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Question 5: XXXXXX

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Average Clinical Trial Trajectory

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Preclinical

Basic research

Drug discovery

Preclinical research

Average duration:

1-6 years

Phase 1

Typically enrolling 20-100 volunteers or mildly affected patients

Measures safety and investigates possible side effects of treatment

Average duration:
Typically up to 1 year

Phase 2

Approximately 70% of all new drug research reaches Phase 2

Typically involves several hundred patients with varying levels of disease severity.

Measures the effectiveness of the drug and checks for side effects

Average Duration: Several Months to 2 years

Phase 3

Approximately 33% of new drug research reaches Phase 3

Tests the largest number of patients possible (in relation to the disease size)

Average Duration: 1 – 4 years

Approval & commercialisation

Only 25 to 30% of treatments are approved.

After completing Phase 3 a pharmaceutical company can move forward with a New Drug Application (NDA) or a BLA in the US and a marketing authorisation approval (MAA) in the EU

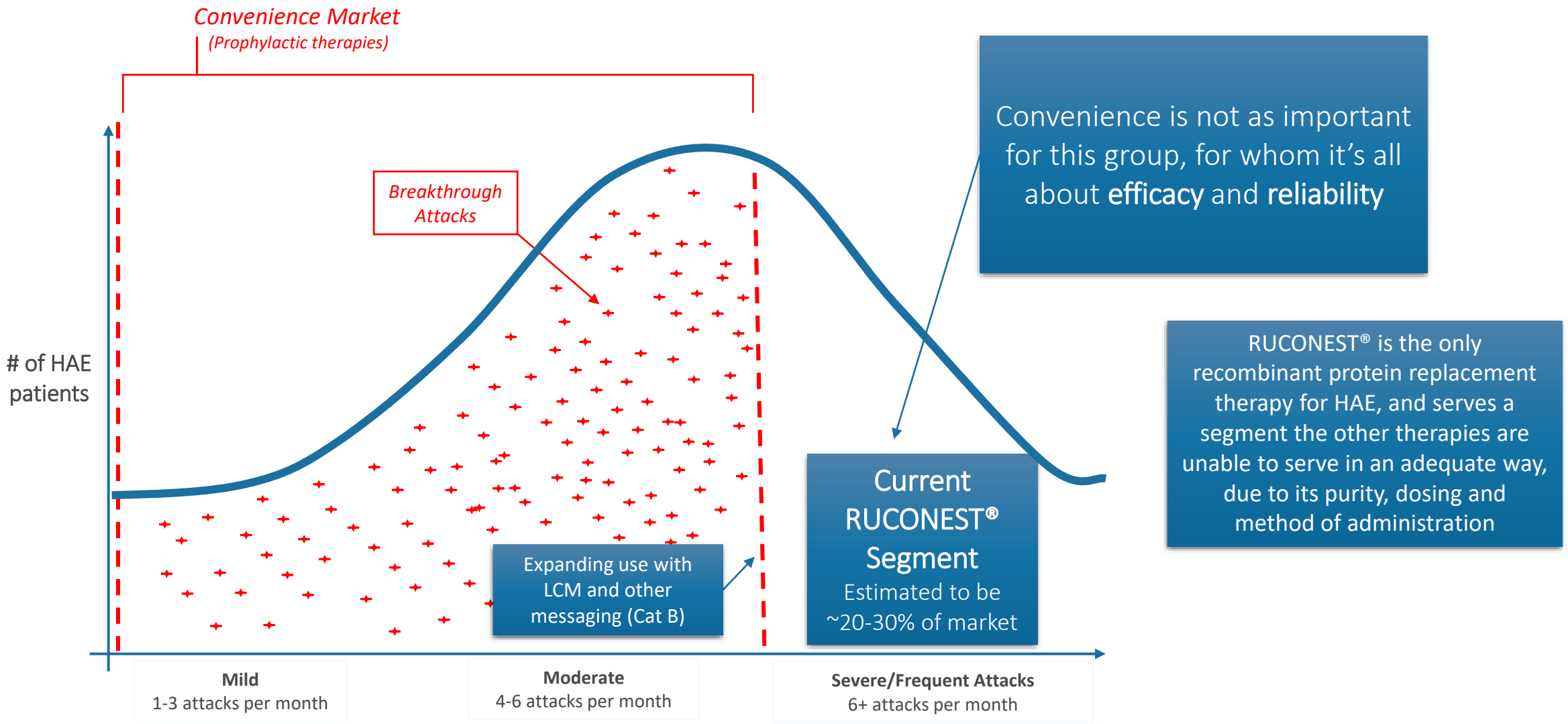
Total Average Duration:

6 – 7 years

Variation in this trajectory:

- Orphan/rare disease
- Product availability
- New drug or a new indication for an existing drug

RUCONEST®: Patient Segmentation in HAE



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

Clinical trials: COVID-19 (Basel + Mexico & Brazil)

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Conestat Alfa in the Prevention of Severe SARS-CoV-2 Infection in Hospitalized Patients With COVID-19

- **Investigator initiated study:** University Hospital Basel, Basel, Switzerland
- **Led by:** Prof. Michael Osthoff

- **Start date:** August 6, 2020 in Basel, Switzerland. Study to expand to centers in Mexico and Brazil shortly.
- **Recruiting status:** Recruiting
- **Estimated end date:** H1 2021

- Phase 2 trial
- 120 participants
- Randomised, open-label, parallel-group, controlled, multi-center trial

- **Treatment:** Initial double dose followed by 8 single doses: 20 vials over 72 hours
- **Outcome measured over 14 days by**: Disease severity, time to clinical improvement, number of patients not requiring invasive or non-invasive ventilation and Acute Lung Injury.

ClinicalTrials.gov Identifier: NCT04414631

Clinical trials: COVID-19 (US)

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Prevention of Severe SARS-CoV-2 Infection in Hospitalized Patients With COVID-19

- **Pharming Technologies B.V. study**

- **Estimated start date:** September 15, 2020 in multiple centers in the US
- **Recruiting status**: Recruitment open
- **Estimated end date:** H1 2021

- Phase 2 trial
- 120 participants
- Randomised, open-label, parallel-group, controlled, multi-center trial, pilot in the United States

- **Treatment:** Patients receive 8 single doses (2 vials) with a maximum of 16 vials over 96 hours.
- **Outcome measured over 14 days by**: Disease severity, time to clinical improvement, number of patients not requiring invasive or non-invasive ventilation and Acute Lung Injury.

ClinicalTrials.gov Identifier: NCT04530136

Clinical trials: leniolisib (APDS)

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Study of Efficacy of Leniolisib in Patients With APDS/PASLI + Extension study

- Novartis Pharmaceuticals in partnership with Pharming Technologies B.V. study
- **Start date:** August 24, 2015
- **Recruiting status:** Recruiting
- **Estimated end date:** June 8, 2021

Phase 2/3: 2-part trial with 36 participants:

i. Open-label, non-randomized to establish safety and pharmacokinetics.

ii. Randomized, subject, investigator blinded, placebo-controlled study to determine optimal dose in target population

Treatment:

- **Part 1** was with-in patient dose escalation with leniolisib 10, 30, 70mh bid.
- **Part 2** is randomized placebo-controlled with leniolisib 70mg bid and matching placebo.

Outcome: over 12-week timeframe

- **Part 1** safety, tolerability, dose PD and PK/PD relationship of leniolisib in patients with APDS
- **Part 2** Assess clinical efficacy
  - Change from baseline assessment of organ volumes determined by MRI/CT imaging
  - Change from baseline in percentage of naïve B cells out of total B cells

Extension study to investigate long-term safety, tolerability, efficacy and pharmacokinetics:

- **Start date:** September 9, 2016
- **Recruiting status:** Recruiting
- **Estimated end date:** September 1, 2026
  - Follow-up extension
  - Open-label, non-randomised
  - Treated daily with 140mg leniolisib

ClinicalTrials.gov Identifier: NCT02435173 & NCT02859727

Clinical trials: Pre-eclampsia

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Study of Efficacy in the treatment of patients with Pre-Eclampsia with rhC1 INH (conestat alfa).

- Pharming Technologies B.V. study

- **Start date:** on hold due to COVID-19
- **Recruiting status:** Recruiting
- **Estimated end date:** H2 2022

- **Trial locations:**
  - University hospital Groningen in the Netherlands
  - University hospital Adelaide in Australia
  - Private Hospitals in Mauritius

- Phase 1/2 trial
- 30 participants with mild/late stage pre-eclampsia
- Multi-center, open-label, parallel-group, controlled study

- **Treatment:** 50 U/kg twice weekly until delivery + Standard of Care.
- **Outcome measured by**: Time from start of treatment to delivery, proportion of patients reaching gestation of week 37 and change from baseline of urine protein levels.

Clinical trials: Acute Kidney Injury

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Study to evaluate the efficacy of conestat alfa compared to placebo after PCI in NSTEMI patients.

- Following positive results from a Phase 2 investigator-initiated study of RUCONEST® in a double-blind, placebo-controlled clinical trial in patients at risk of nephropathy resulting from contrast-enhanced examinations.

- **Pharming Technologies B.V. study**

- **Start date:** H2 2020
- **Recruiting status:** Not yet recruiting
- **Estimated end date:** H2 2021

- Phase 2 trial
- 220 participants
- Double-blind, randomized, controlled study

- **Treatment:** 50 U/kg Conestat or 100 U/kg conestat alfa or Placebo.
- **Outcome measured by**: The peak increase of urinary NGAL within 24 hours after PCI. With a 6 month follow up.