

# Pharming Group N.V.

Pan European small/ midcap Conference

JP Morgan

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# Forward Looking Statements



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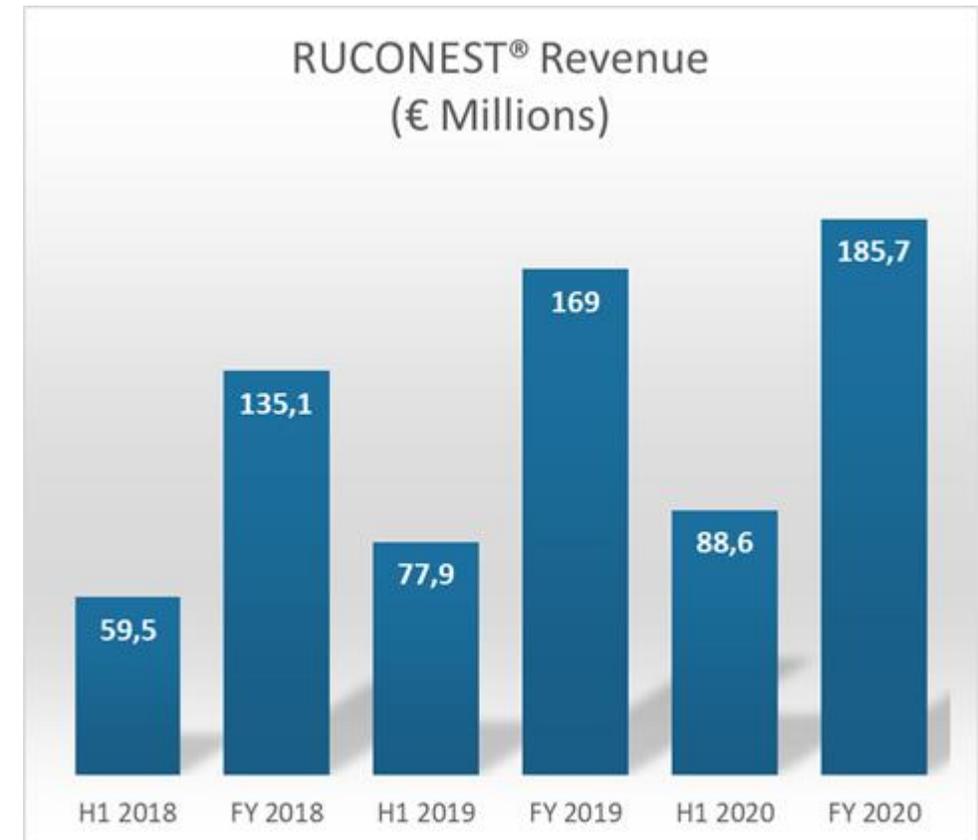
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# Company Overview

- ◆ Established in 1988, based in the Netherlands with 250+ employees
- ◆ Listed on the Nasdaq: **PHAR** & Amsterdam stock exchange: **PHARM**
- ◆ Rare and ultra-rare disease development and commercialisation:
  - 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 in follow-on indications
- ◆ Late-stage in-licenced product: **leniolisib**, for the treatment of Activated Phosphoinositide 3-kinase Delta Syndrome (APDS)



- Record revenue in FY 2020, 9.9% increase to €185.7m (FY 2019: €169.0m)
- Gross profits in FY 2020 increased 11.8% to €165.1m (FY 2019: €147.7m)
- Operating profit in FY 2020 increased 10.7% to €67.4m (FY 2019: €60.9m)
- Net profit in FY 2020 decrease of 9.8% to €32.7m (FY 2019:€36.2m), reflecting negative currency effects of €12.6m
- Strengthened cash position at year end to €168.3m (cash on 31 December 2019: €68.9m)
- Successfully placed €125m 3% senior unsecured convertible bonds due 2025
  - Proceeds used to redeem the remaining \$55.6m loan with Orbimed Advisors
  - Balance of proceeds to support expansion of commercialization and manufacturing infrastructure, launch of leniolisib and acquisitions/in licensing opportunities





Investing for long-term sustainable revenue growth

# Three-pillar strategy for growth

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

- Fully commercialize 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 APDS
- 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

- RUCONEST® approved for the treatment of acute HAE attacks in adults and adolescents in the US
- Patients' treatment plans (if on prophylaxis) include break-through medication
  - New prophylactic treatments offer better attack reduction rates than previous IV plasma-derived C1INH prophylaxis treatment
  - According to published data: approximately half of the patients using new prophylaxis treatments continue to have breakthrough attacks, some frequently, and are in need of regular use of breakthrough medication
  - Although kallikrein/bradykinin inhibitors block the main pathway for symptomatology, an uncontrolled breakthrough attack can occur and become serious if no C1INH therapy is available
- Increasing recognition for prophylaxis patients to have effective and reliable C1INH treatment for breakthrough attacks at hand
  - Growth opportunity for RUCONEST® for treatment of breakthrough attacks associated with prophylaxis products

# Investment to increase capacity due to strong demand

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- Investment in de-risking and upscaling of production capacity
  - Pharming received both EMA and FDA approval for its new production facility of starting material for RUCONEST®
  - Third facility under construction to safeguard future growth in HAE supplies
  - Plans for a larger fourth facility to manufacture our other pipeline products
  - Building downstream processing facility to expand in-house processing capacity
- Patient numbers in potential new indications are much larger than in HAE
- Re-developing rhC1INH from cattle to meet future demand for large indications
- Funded from current cash generation

- Clinical trial for rhC1INH in pre-eclampsia and acute kidney injury temporarily halted due to COVID-19
- Clinical trials for rhC1INH in patients hospitalized with confirmed SARS-CoV-2 infections
  - University Hospital of Basel, Basel, Switzerland
    - Results from compassionate use study in five patients
      - Published in *Frontiers in Immunology*
    - Multinational, randomized, controlled, investigator-initiated study of up to 150 patients in Switzerland and expanded across the country and into Brazil and Mexico
      - Recruitment ongoing
  - Valley Hospital in Ridgewood, New Jersey, US
    - Randomized, open-label, parallel-group, controlled, clinical trial in up to 120 participants across centers in the US
      - Recruitment ongoing

## APDS market

- Activated PI3 kinase delta syndrome (APDS) is ultra-rare primary immunodeficiency (PID)
  - Caused by autosomal dominant mutations
  - Increased activity of phosphoinositide-3-kinase  $\delta$  (PI3K $\delta$ ) leads to malfunctioning B-(immune) cells, symptoms include; recurrent respiratory infections, organomegaly, malignancies and auto-immunity
  - 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%)
- Current diagnosis and treatment options for APDS
  - Often misdiagnosed
  - Treatment limited to supportive therapies; antibiotics, immunoglobulin replacement therapy
  - No approved therapy for treatment
  - Genetic test only definitive diagnosis

## leniolisib program

- 'navigateAPDS' partnership in the US and Canada
  - Collaboration with Invitae for a commercially available genetic test
- leniolisib
  - Potent, selective PI3K $\delta$  inhibitor
  - Treats the root cause of APDS
  - Orally bioavailable
  - Direct PK/PD relationship observed
  - Currently in registration-enabling pivotal study
  - Expected headline data H2 2021 with potential launch H2 2022
- Orphan drug designation approved by the European Commission
  - Previously granted Orphan Drug Designation by the FDA in January 2018

# 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.

The impact of COVID-19 on the operations of the business is summarized below:

- No impact on the upscaling or continued production of RUCONEST® to date, despite disruptions in supply chains for consumables used in production
- No impact on the availability or distribution of RUCONEST® to HAE patients
- The recruitment of new patients in ongoing clinical trials halted as result of COVID-19 priorities and disruptions in supply chains of test materials; patients already incorporated into ongoing clinical trials are continuing to receive treatment

- Continued growth in revenues from sales of RUCONEST<sup>®</sup>, mainly driven by the US and expanded EU operations, subject to the progression of the COVID-19 pandemic, with quarterly fluctuations in revenues expected, as a result of the ongoing effects of the pandemic on access to customers and phasing of ordering patterns.
- Maintenance of positive net earnings during the year, we therefore do not expect to require additional financing to maintain the current business.
- Investments in acquisitions and in-licensing of new development opportunities and assets, as these occur
- Continued investment in the expansion of production of RUCONEST<sup>®</sup> and production of leniolisib
- Investment in:
  - Ongoing registration-enabling study for leniolisib and pre-marketing activities
  - Ongoing clinical trials for rhC1INH
  - Additional development activities
- Continued close monitoring of the ongoing COVID-19 pandemic and the potential impact on the business

No further specific financial guidance for 2021 is provided.

As previously announced, as of 1 January 2021, the Company changed its reporting currency from Euro to US dollar.

[www.pharming.com](http://www.pharming.com)

ENXTAM: PHARM

Nasdaq: PHAR

Bloomberg: PHAR.AS