# Pharming Group N.V.

Full Year Results 2019

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# Forward Looking Statements

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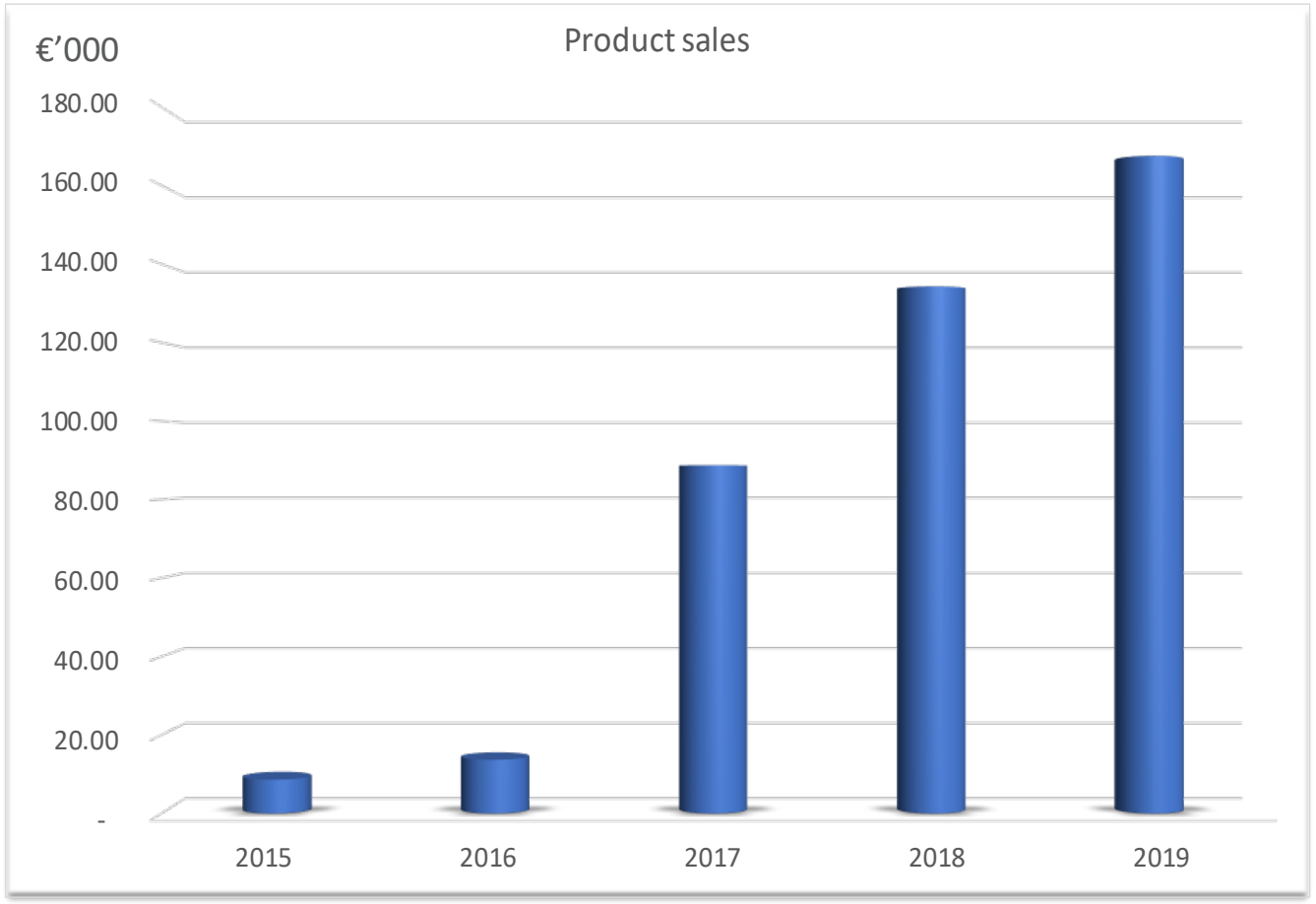
# Full year results 2019

# Strong Execution of Commercial Strategy

- Continued growth from existing and new patients using RUCONEST® to treat acute HAE attacks, and increasingly also as a preferred therapy for breakthrough HAE attacks while using prophylactic therapies



# RUCONEST® in a changing HAE landscape

- Multiple treatment options provide better management of HAE
  - New treatments offer better attack reduction rates than previous IV plasma-derived C1INH prophylaxis treatment
  - Competitive pressure in prophylaxis segment continues to increase, with potential approval of the first oral product in December 2020
  - Most patients using (new) prophylaxis treatments continue to have breakthrough attacks, some frequently, according to published data
  - Kallikrein only inhibitors block the main pathway for symptomatology, so an uncontrolled breakthrough attack can be serious if no C1INH therapy is available
- There is therefore a recognised need for prophylaxis patients to have an effective and reliable C1INH treatment for breakthrough attacks at hand (as opposed to on demand or acute therapy)
  - Increased growth opportunity for RUCONEST® for treatment of breakthrough attacks associated with current and new prophylaxis products
- In addition, RUCONEST® continues to be a preferred treatment for acute HAE attacks as well as for severely-affected patients

# Investing for long-term sustainable revenue growth

# Pharming: Built to Grow


- Pharming has all the capabilities needed for sustainable, high growth
- Today, we are a profitable, growing international biotech, with one launched product with multiple opportunities, ~$750M market capitalization, fully integrated from primary research through production platform to commercialization
- Tomorrow, we will be a global pharmaceutical company with multiple products with blockbuster potential, both in rare and specialty diseases and in large currently unmet indications

# Investment to increase capacity due to strong demand

- Underlying demand for RUCONEST® increasing further in both US and rest of the world
  - New second production facility in Netherlands already validated by EMA and under review with FDA
  - EMA approval of new facility has eased supply pressure in Europe, although validation of manufacturing process for new versions of RUCONEST® and clinical trials to validate other routes of administration remain slow until supplies are available
  - 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
  - Completed investment in fill & finish partner, which manufactures the final sealed vials of RUCONEST®
  - 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 and acute kidney injury are much larger than for HAE
- Redeveloping rhC1INH from cattle as a new variant to meet future demand for these such large indications
- 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 that will indicate need for additional larger downstream facilities
- 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, with further increased sales if leniolisib is approved

# New opportunities for rhC1INH

- Clinical trial for rhC1INH in preeclampsia is ongoing, with preliminary safety data due later this year
- Clinical trial for rhC1INH in acute kidney injury in patients undergoing percutaneous coronary interventions such as stent insertions and valve replacements is expected to dose its first patient soon
- Interest from investigators in using rhC1INH to study its effects on mitigating various conditions causing dangerous immune system over-reactions called cytokine storms, including indications such as adult respiratory distress syndrome (ARDS)
- C1INH does not cure anything, but as the only natural inhibitor of the complement system and the relevant cytokines, it could potentially play a major part in large severe indications

# Leniolisib and APDS Background

- 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 all PIDs

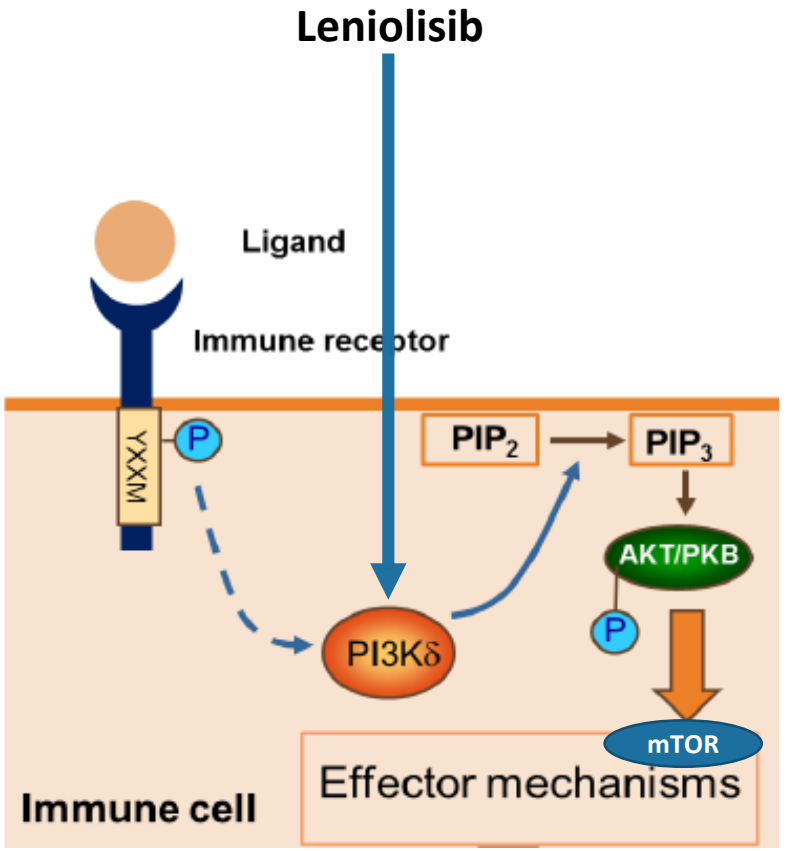
- Activated PI3 kinase delta syndrome (APDS) is a primary immunodeficiency
  - Caused by autosomal dominant mutations
  - Increased activity of phosphoinositide-3-kinase δ (PI3Kδ)
  - Estimated prevalence 1-2/million
  - More than 240 reported in literature
  - Screening in subset of PID patients has 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 Treatment Options

- 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



# Other Investments

## Pompe disease

- Producing α-glucosidase for studies to enable an IND towards end of 2021
- Subject to regulatory authority inputs; study design now enlarged, with a multiple ascending dose Phase Ib/II trial in patients
- This means starting later (due to amount of material required from current small-scale manufacture) but finishing at same time as previous single-dose Phase I start plan
- We believe the drug will be less immunogenic than current therapy option

# Coronavirus Covid 19

- No major issue for Pharming
- No production or clinical implications for our manufacturing or pipeline yet
- No impact on patients' demand for or use of RUCONEST®
- As most of our clinical activity is for rare diseases or indications where there is no current therapy, we expect limited impact on clinical trial recruitment

# Financial statements

# Income Statement – Operating Profit

| Amounts in € '000 | 2019 | 2018 |
|---|---|---|
| **Revenues** | **169,022** | **135,130** |
| **Costs of sales** | **(21,355)** | **(22,180)** |
| **Gross profit** | **147,667** | **112,950** |
| **Other income** | **435** | **684** |
| Research and development | (32,940) | (28,882) |
| General and administrative | (14,341) | (12,221) |
| Marketing and sales | (39,914) | (34,539) |
| **Costs** | **(87,195)** | **(75,642)** |
| **Operating result** | **60,907** | **37,992** |

# Delivering Record Revenues

- Record revenues – increase of 25% to €169.0m in 2019 (2018: €135.1m)
  - Quarter 4 ahead of Quarter 3 for the first time: € 45.7m in Q4 2019 (Q3 2019: €45.5m)
- US product sales increased 29% to €162.7m in 2019 (2018: €126.6m)
- EU& RoW sales down slightly due to supply pressure and clawback caps, from €7.6 million in 2018 to €4.9 million in 2019
  - This should be reversed in 2020 now that former Sobi territory is back in Pharming control
  - Replacement of Sobi business now well under way in approximately 15 of the returned countries
  - Sobi transaction expected to be accretive to earnings in 2020
- Gross Margin improved slightly due to favourable changes in sales mix and intermittent competitor shortages in EU markets: €147.7 million in 2019 (87%) vs €113.0 million in 2018 (84%)

# Increased Investment in Growth

- Operating profit increased 60% to €60.9 million in 2019 (2018: €38.0 million)
  - Reflects the improvement in gross margin, and better cost controls, but also increased operating costs needed to prepare for new clinical studies
- Net profit increased by 45% to €36.2 million in 2019 (2018: €25.0 million)
- Increased investment in pipeline and infrastructure to support long-term growth
  - Increased expenditure in H2 2019 (compared to H1) on pre-eclampsia and acute kidney injury studies, production of α-glucosidase product for Pompe disease and capacity improvements
  - Investment in our fill & finish partner Bioconnection to support capacity expansion
  - Acquisition of a license to Leniolisib from Novartis for $20 million upfront
  - Re-acquisition of rights to RUCONEST® in the rest of Europe from Sobi for €7.5 million

# Income Statement – Net Result

| Amounts in € '000 | 2019 | 2018 |
|---|---|---|
| **Operating result** | **60,907** | **37,992** |
| Fair value gain (loss) on revaluation derivatives | (209) | (495) |
| Other financial income | 1,011 | 18 |
| Other financial expenses | (15,259) | (36,658) |
| **Financial income and expenses** | **(14,457)** | **(37,135)** |
| Share of net profits of associates using the equity method | 229 | - |
| **Result before income tax** | **46,679** | **857** |
| Income tax credit (expense) | (10,484) | 24,136 |
| **Net result for the year** | **36,195** | **24,993** |
| **Attributable to:** | | |
| Owners of the parent | 36,195 | 24,993 |
| **Total net result** | **36,195** | **24,993** |
| Basic earnings per share (€) | 0.058 | 0.041 |
| Fully-diluted earnings per share (€) | 0.054 | 0.038 |

# Balance Sheet – Assets

| Amounts in € '000 | 2019 | 2018 |
|---|---|---|
| **Non-current assets** | | |
| Intangible assets | 78,309 | 52,435 |
| Property, plant and equipment | 8,553 | 8,402 |
| Right of Use assets | 5,979 | - |
| Long-term prepayments | - | 2,006 |
| Deferred tax assets | 30,933 | 35,082 |
| Investments acounted for using the equity method | 5,307 | - |
| Restricted cash | 2,268 | 1,204 |
| **Total non-current assets** | **131,349** | **99,129** |
| **Current assets** | | |
| Inventories | 14,467 | 17,315 |
| Trade and other receivables | 26,807 | 17,814 |
| Cash and cash equivalents | 66,299 | 80,311 |
| **Total current assets** | **107,573** | **115,440** |
| **Total assets** | **238,922** | **214,569** |

# Balance Sheet – Liabilities

| Amounts in € '000 | 2019 | 2018 |
|---|---|---|
| **Equity** | | |
| Share capital | 6,313 | 6,215 |
| Share premium * | 392,266 | 387,525 |
| Legal reserves | 4,043 | (590) |
| Accumulated deficit | (297,943) | (331,399) |
| **Shareholders' equity** | **104,679** | **61,751** |
| **Non-current liabilities** | | |
| Loans and borrowings * | - | 37,267 |
| Deferred tax liabilities | 2,343 | 87 |
| Contract liabilities | - | 667 |
| Finance lease liabilities | 4,363 | 164 |
| Other financial liabilities | 17,081 | 32,034 |
| **Total non-current liabilities** | **23,787** | **70,219** |
| **Current liabilities** | | |
| Loans and borrowings * | 45,590 | 35,235 |
| Contract liabilities | - | 800 |
| Derivative financial liabilities * | 268 | 228 |
| Trade and other payables | 44,817 | 28,589 |
| Finance lease liabilities | 1,946 | 263 |
| Other financial liabilities | 17,835 | 17,484 |
| **Total current liabilities** | **110,456** | **82,599** |
| **Total equity and liabilities** | **238,922** | **214,569** |

# Cash Flow – Operating Activities

| Amounts in €'000 | 2019 | 2018 |
|---|---|---|
| **Operating result** | **60,907** | **37,992** |
| ***Non-cash adjustments:*** | | |
| Depreciation, amortisation, impairment | 5,177 | 6,559 |
| Accrued employee benefits | 3,825 | 3,270 |
| Deferred license fees | (1,467) | (804) |
| **Operating cash flows before changes in working capital** | **68,442** | **47,017** |
| ***Changes in working capital:*** | | |
| Inventories | 3,067 | 1,019 |
| Trade and other receivables | (9,562) | (6,554) |
| Payables and other current liabilities | 15,433 | 1,391 |
| **Total changes in working capital** | **8,938** | **(4,144)** |
| Changes in non-current assets, liabilities and equity | (2,006) | (1,098) |
| **Cash generated from (used in) operations before interest and taxes** | **75,374** | **41,775** |
| Interest received | 1,011 | 18 |
| Income taxes paid | (3,284) | (1,417) |
| **Net cash flows generated from (used in) operating activities** | **73,101** | **40,376** |

# Cash Flow – Overall

| Amounts in €'000 | 2019 | 2018 |
|---|---|---|
| **Net cash flows generated from (used in) operating activities** | **73,101** | **40,376** |
| Capital expenditure for property, plant and equipment | (2,362) | (2,496) |
| Investment in Intangible assets | (9,944) | (1,273) |
| Investment in Associates | (2,503) | |
| Acquisition of license | (17,908) | - |
| **Net cash flows used in investing activities** | **(32,717)** | **(3,769)** |
| Repayment of loans and borrowings | (31,144) | (15,137) |
| Payments of contingent consideration | (17,634) | - |
| Redemption of Bonds | - | (2,257) |
| Interest on loans | (8,680) | (11,063) |
| Proceeds of equity and warrants | 2,778 | 10,496 |
| **Net cash flows generated from (used in) financing activities** | **(54,680)** | **(17,961)** |
| **Increase (decrease) of cash** | **(14,296)** | **18,646** |
| Exchange rate effects | 1,348 | 2,876 |
| Cash and cash equivalents at 1 January | 81,515 | 59,993 |
| **Total cash and cash equivalents at 31 December** | **68,567** | **81,515** |

# Outlook for Full Year 2020

# Outlook for 2020

For the remainder of 2020, the Company expects:

- Continued growth in revenues from sales of RUCONEST®, mainly driven by the USA and expanded European 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 Rest of the World.
- Investment in the ongoing clinical trials for pre-eclampsia and acute kidney injury, and support for investigators wishing to explore additional indications for RUCONEST®
- Investment in the continuing registration-enabling study for leniolisib for APDS, leading to headline data at the end of the year or early in 2021.
- Investment in preparing for further clinical trial programs for RUCONEST® in acute treatment of HAE, initially by means of the development of a small volume version for intramuscular injections and research into applicability of pain- free delivery methods for prophylaxis of HAE
- Investment in IND enabling studies for α-glucosidase in Pompe disease and preclinical development of the new recombinant α-galactosidase candidate for Fabry's disease
- Investment in other new development opportunities and assets as these occur

## Tickers:

- ENXTAM: PHARM
- Bloomberg: PHAR.AS