

Pharming Group N.V.

Q1 Results 2021

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Forward looking statement

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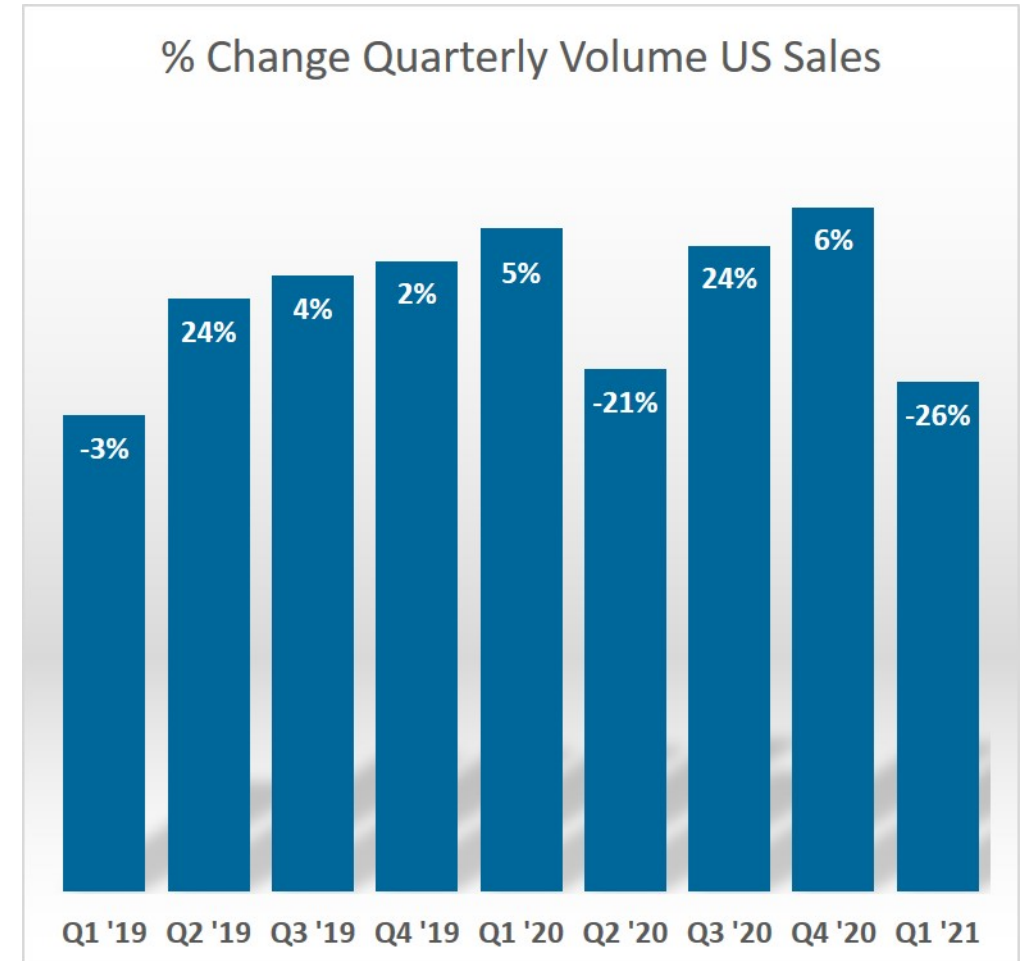
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- Revenue for Q1 2021 decreased 20% to \$43.6 million (Q1 2020: \$54.5 million)
 - Similar to Q2 2020, this quarter results were impacted by effects of another COVID-19 surge during November, December and into 2021
 - Patients stocking RUCONEST® during surge in Q4 2020 led to:
 - Lower prescription refill rates by patients still using additional RUCONEST® stock
 - Closure of physician offices led to:
 - Reduction in routine and diagnostic patient visits
 - Reduction in new patient enrollments in the first part of Q1 2021
 - Slower than normal renewals of annual prescriptions
 - Towards the end of Q1 2021, these trends started to reverse, with a significant increase in new patient enrollment
- Net profit of \$8.5 million decreased 9% (Q1 2020: \$9.3 million)
- Positive cashflows from operations of \$7.1 million in Q1 2021

Expected return to growth in Q2 2021

- Revenue in US decreased due to COVID-19 surge
 - Total value of HAE market is increasing again (2020) driven by increasing use of prophylaxis
 - No major shifts in volume and market shares observed between RUCONEST® and competitors
 - Return to growth in Q2 2021 and beyond expected
- Revenues in Europe and RoW remained stable
 - Continued build out of EU commercial infrastructure and expansion into new territories following re-acquisition of EU rights for RUCONEST® from Sobi



- RUCONEST® approved for the treatment of acute HAE attacks in adults and children
- 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; gradual growth of prophylaxis segment
 - According to published data: depending on product; 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
 - Gradual change and extension of patient population as result of increasing use of RUCONEST® for treatment of breakthrough attacks associated with prophylaxis products



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



Progress towards an anticipated launch of leniolisib
for APDS in Q4 2022

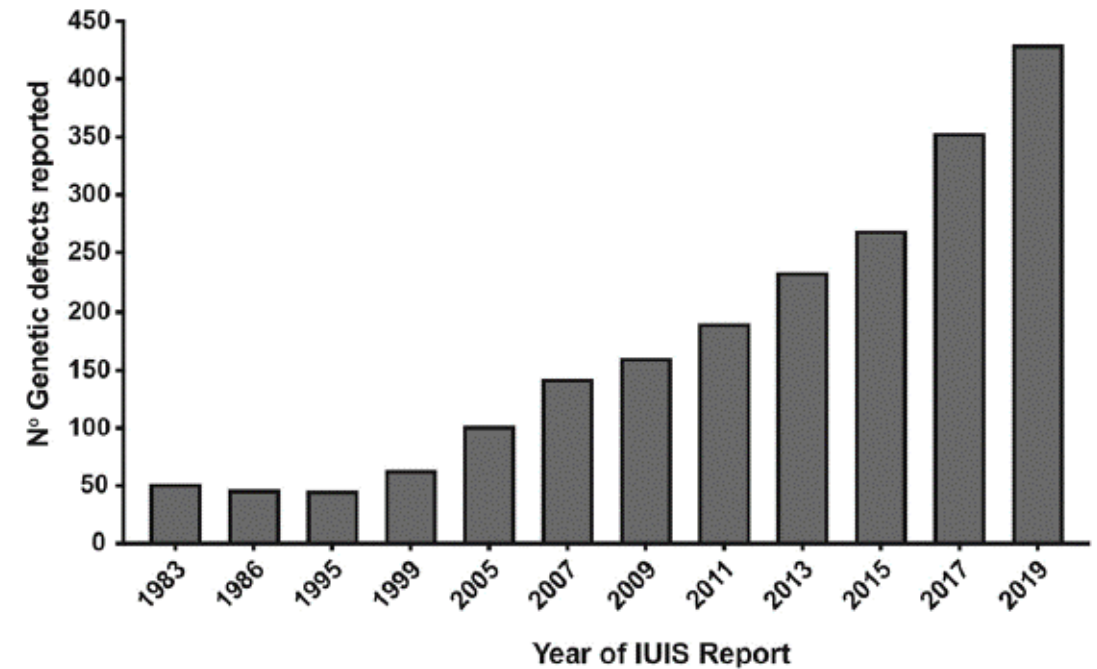
APDS is a primary immunodeficiency (PID)

Primary immunodeficiencies:

- Prevalence: ~1 in 1200
- Gene mutations: >400
- Clinical presentation: Highly variable
- There is a growing understanding that PID patients are not just about infections – they underlie many types of autoimmunity and a wide range of diseases¹⁻³

Greater understanding of PID's is revealing a larger patient population³

The number of identified PID's is growing steadily¹



1. Tangye SG, et al. *J Clin Immunol*. 2020;40(1):24-64. 2. McCusker C, et al. *Allergy Asthma Clin Immunol*. 2018;14(Suppl 2):61. 3. Chan AY, et al. *Front Immunol*. 2020;11:239.; IUIS: International Union of Immunological Societies

Significant market opportunity

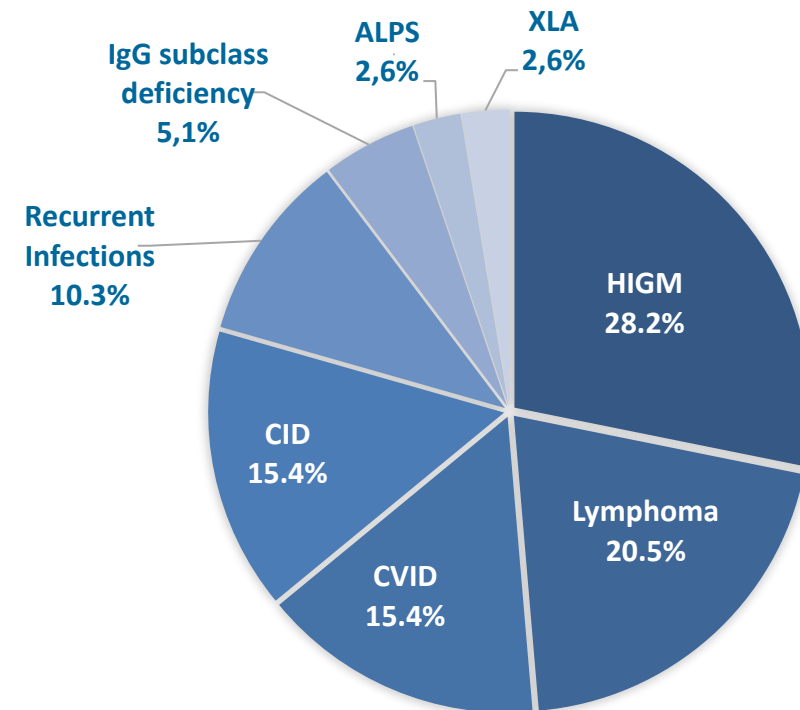
APDS was fully characterized in 2013¹⁻⁴

- Caused by autosomal dominant variations in one of two genes, leading to APDS1 or APDS2
- Results in hyperactivation of phosphoinositide-3-kinase δ (PI3K δ) which suppresses and dysregulates the immune system
- Balanced PI3K δ signaling is essential for normal immune function^{5,6}

Prevalence:

- 1.5/million (estimated)^{7,8}, >240 reported in literature⁹
- Screening in subset of patients with PI found rates: 5/669 (1%)¹⁰ and 17/184 (9%)¹

Diagnostic criteria: genetic test (commercially available)¹¹



Cases of APDS are often diagnosed as HIGM or lymphoma⁹

ALPS, autoimmune lymphoproliferative syndrome; CID, combined immune deficiency; CVID, common variable immunodeficiency; HIGM, hyperimmunoglobulin M; XLA, X-linked agammaglobulinemia.

1. Angulo I, et al. Science. 2013;342(6160):866-871. 2. Lucas CL, et al. Nat Immunol. 2014;15:88-97. 3. Lucas CL, et al. J Exp Med. 2014;211(13):2537-2547. 4. Deau MC, et al. J Clin Invest. 2014;124(9):3923-3928. 5. Okkenhaug K, Vanhaesebroeck B. Nat Rev Immunol. 2003;3:317-330. 6. Fruman DA, et al. Cell. 2017;170(4):605-635. 7. Orphanet. https://www.orpha.net/consor/cgi-bin/OC_Exp.php?Ing=EN&Expert=397596. 8. . DOF, Pharming Healthcare, Inc. 2019. 9. Jamee M, et al. Clin Rev Allergy Immunol. 2019;May 21. 10. Elgizouli M, et al. Clin Exp Immunol. 2016;183(2):221-229. 11. Chinn IK, et al. J Allergy Clin Immunol. 2020;145(1):46-69.

Leniolisib: potential to address unmet needs in APDS

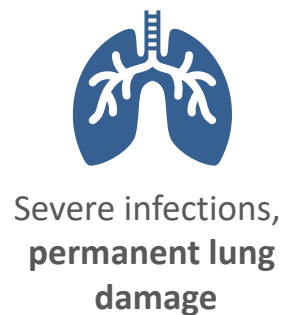
Burden of APDS¹⁻⁴

- Estimated > 1,350 patients (500 US, 675 EU, 190 Japan) live with APDS (based on prevalence)
- APDS patients are characterized across all global regions
- Years spent undiagnosed or misdiagnosed, seeing 4-5 specialists
- Symptoms begin in childhood & disrupt school and social development
- Significant impact on QoL:
 - Surgical interventions are common
 - Care typically managed by >4 doctors
 - Depression and fatigue significantly impact QoL

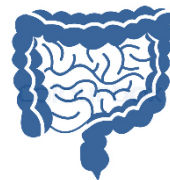
Current treatment options for APDS⁵

- Symptomatic therapies (e.g., antibiotics, steroids)
- Immunoglobulin replacement therapy (IRT) infusions
- mTOR inhibitors (e.g., sirolimus, rapamycin) off-label for lymphoproliferative symptoms only
- Hematopoietic stem cell transplantation

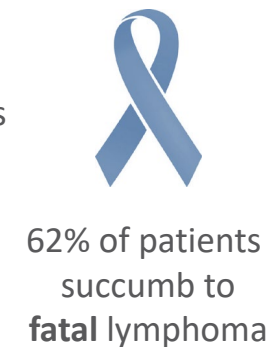
Often used together

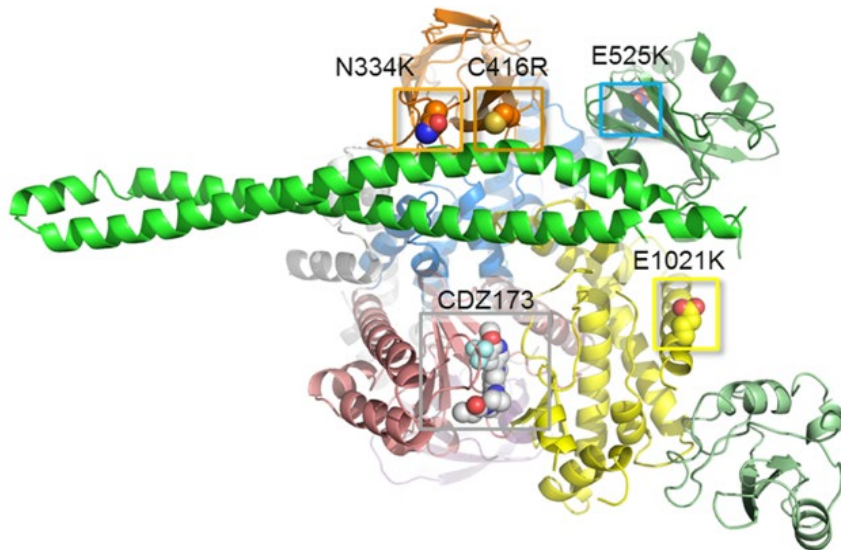


GI disease so severe it impairs growth



Autoimmunity including severe anemias & bleeding disorders





X-ray crystallography model of lenolisib (CDZ173) bound to catalytic subunit p110δ with regulatory subunit p85α overlaid and mutations highlighted (pdb 2y3a) (modeling by H. Moebitz)

Broad impact on lymphocytes¹

- CD4+ T cells
- CD8+T cells
- B cells
- NK cells

Therapeutic Area

APDS : Activated PI3K Delta Syndrome

US Regulatory Target

Year End
Q4/2022

Topline Data Target
Q4/2021

Leniolisib^{2,3}

Effective oral selective PI3Kδ inhibitor

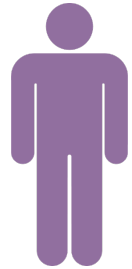
Precision biomarker response demonstrates impact on root cause

Potential to mitigate progression of disease & reduce treatment burden

APDS diagnosis made by a commercially available genetic test⁴

1. Lucas CL, et al. Nat Immunol. 2014;15:88-97. 2. Rao VK, et al. Blood. 2017;130(21):2307-2316. 3. Hoegenauer K, et al. ACS Med Chem Lett. 2017;8(9):975-980. 4. Chinn IK, et al. J Allergy Clin Immunol. 2020;145(1):46-69.

Patient identification process



Access to **leading global physicians** with an integrated clinical network

Cross-therapeutic **Steering Committee & Advisory Boards**

Validated blueprint of the patient journey & **referral pathways**

Sponsored **genetic testing** with access to the growing database of patients with APDS

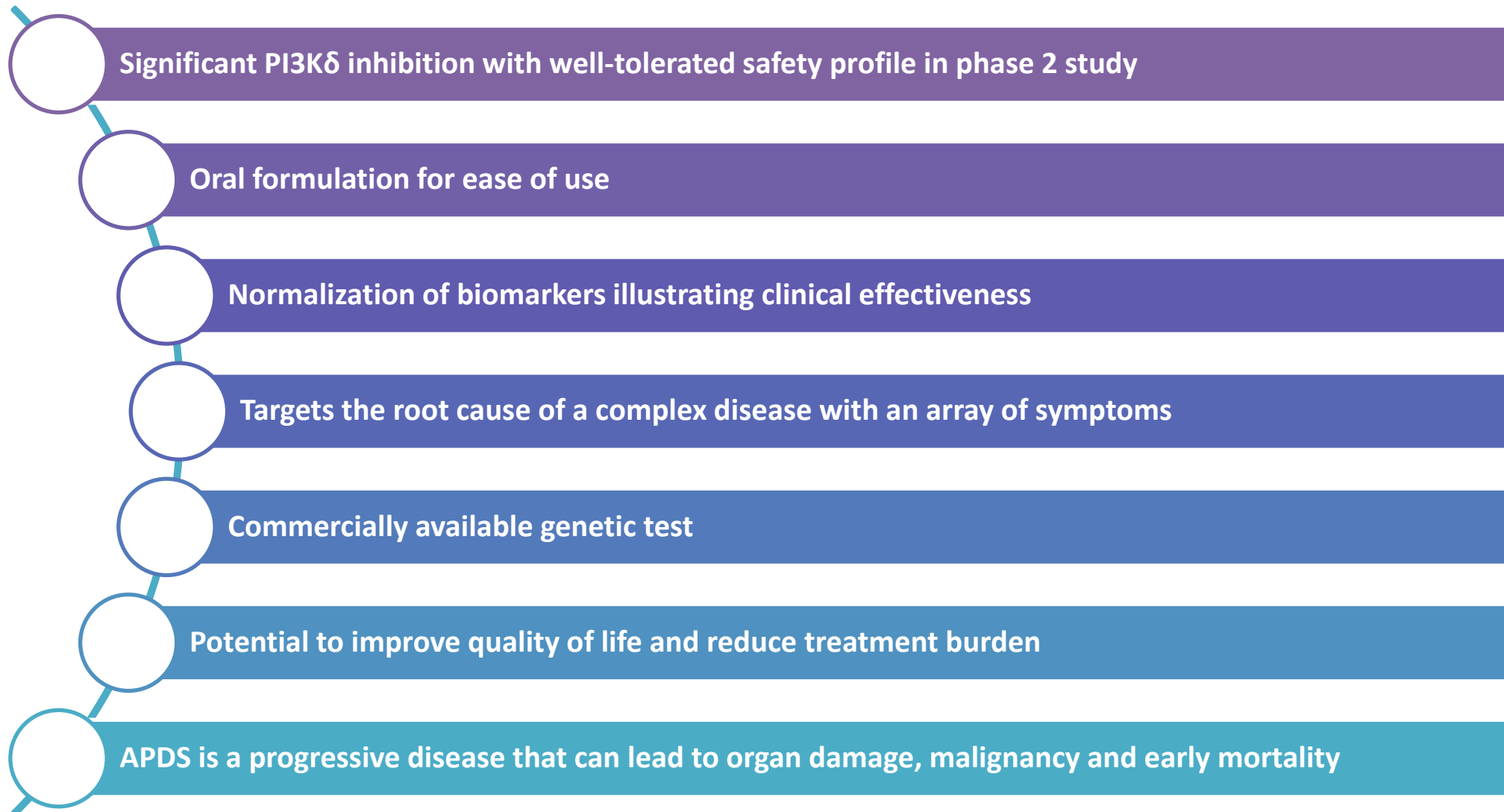
Strategic support to **trace genetic inheritance**



A.I algorithm to identify patients within hospitals, in partnership with KOLs

Database searches to identify patients and understand patient journey

Leniolisib value proposition in APDS





Expansion of rhC1INH franchise for larger indications

- Enrolment has begun for Phase IIb clinical trial for rhC1INH in acute kidney injury after myocardial infarction
 - Acute kidney injury (AKI) defined by rapid onset of renal damage and dysfunction
 - University Hospital of Basel, Basel, Switzerland
 - Double-blind, randomized controlled study in up to 220 patients
- Ongoing trials for rhC1INH inpatients hospitalized with confirmed SARS-CoV-2 infections
 - University Hospital of Basel, Basel, Switzerland
 - 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
- Trial for rhC1INH in pre-eclampsia temporarily halted due to COVID-19



Financial Review

Financial Summary

| <i>Amounts in US \$m except per share data</i> | Q1 2021 | Q1 2020 | % Change |
|-------------------------------------------------------------|----------------|----------------|-----------------|
| <i>Consolidated Income Statement</i> | | | |
| Revenues | 43.6 | 54.5 | -20% |
| Gross profit | 38.7 | 48.5 | -20% |
| Operating result | 6.3 | 21.5 | -71% |
| Finance cost, net | 6.6 | -7.8 | -184% |
| Income tax expense | -4.3 | -4.4 | -3% |
| Net result | 8.5 | 9.3 | -9% |
| <i>Consolidated Balance Sheet</i> | | | |
| Cash & marketable securities (Including restricted cash) | 208.5 | 149.4 | 40% |
| <i>Share Information</i> | | | |
| Basic earnings per share (€) | 0.013 | 0.015 | -12,6% |
| Diluted earnings per share (€) | 0.013 | 0.013 | -0,8% |

Income statement

| <i>Amounts in \$ '000</i> | YTD 2021 | YTD 2020 |
|-------------------------------------------------------------------|-----------------|-----------------|
| Revenues | 43.564 | 54.469 |
| Costs of sales | (4.843) | (5.955) |
| Gross profit | 38.721 | 48.514 |
| Other income | 259 | 267 |
| Other Operating Costs | (32.697) | (27.293) |
| Operating profit | 6.283 | 21.488 |
| Fair value loss on revaluation derivatives | 30 | 134 |
| Other finance income | 8.159 | 409 |
| Other finance expenses | (1.598) | (8.378) |
| Finance cost, net | 6.591 | (7.835) |
| Share of net profits in associates using the equity method | (82) | 15 |
| Profit before tax | 12.792 | 13.668 |
| Income tax expense | (4.269) | (4.418) |
| Profit for the year | 8.523 | 9.250 |
| Basic earnings per share (€) | 0,013 | 0,015 |
| Diluted earnings per share (€) | 0,013 | 0,013 |

Balance sheet – assets

| <i>Amounts in \$ '000</i> | March 31, 2021 | December 31, 2020 |
|--------------------------------------------------|-----------------------|--------------------------|
| Non-current assets | | |
| Intangible assets | 89.943 | 94.083 |
| Property, plant and equipment | 13.093 | 12.226 |
| Right-of-use assets | 8.828 | 9.427 |
| Deferred tax assets | 27.559 | 31.877 |
| Investment accounted for using the equity method | 6.720 | 7.118 |
| Restricted cash | 863 | 510 |
| Total non-current assets | 147.006 | 155.241 |
| Current assets | | |
| Inventories | 21.765 | 21.157 |
| Trade and other receivables | 32.941 | 35.902 |
| Restricted cash | 962 | 995 |
| Cash and cash equivalents | 206.625 | 205.159 |
| Total current assets | 262.293 | 263.213 |
| Total assets | 409.299 | 418.453 |

Balance sheet – liabilities

| <i>Amounts in \$ '000</i> | March 31, 2021 | December 31, 2020 |
|--------------------------------------|-----------------------|--------------------------|
| Equity | | |
| Share capital | 7.195 | 7.163 |
| Share premium | 449.135 | 444.940 |
| Legal reserves | 11.358 | 19.859 |
| Accumulated deficit | (281.328) | (288.527) |
| Shareholders' equity | 186.360 | 183.435 |
| Non-current liabilities | | |
| Convertible bonds | 141.169 | 149.727 |
| Lease liabilities | 7.744 | 8.230 |
| Other financial liabilities | 189 | 212 |
| Total non-current liabilities | 149.102 | 158.169 |
| Current liabilities | | |
| Convertible bonds | 3.062 | 2.040 |
| Derivative financial liabilities | 84 | 181 |
| Trade and other payables | 43.682 | 47.666 |
| Lease liabilities | 1.907 | 1.962 |
| Other financial liabilities | 25.103 | 25.000 |
| Total current liabilities | 73.837 | 76.849 |
| Total equity and liabilities | 409.299 | 418.453 |

| <i>Amounts in \$'000</i> | YTD 2021 | YTD 2020 |
|---------------------------------------------------------------------|-----------------|-----------------|
| Profit before tax | 12.792 | 13.668 |
| Net cash flows generated from (used in) operating activities | 7.061 | 21.832 |
| Capital expenditure for property, plant and equipment | (1.956) | (660) |
| Investment intangible assets | (460) | (210) |
| Investment associate | 398 | 8 |
| Acquisition of license | (547) | (6.077) |
| Net cash flows used in investing activities | (2.565) | (6.939) |
| Repayment on loans and borrowings | - | (54.965) |
| Payment on contingent consideration | - | (20.039) |
| Payment of lease liabilities | (554) | (525) |
| Proceeds of issued convertible bonds | - | 138.124 |
| Transaction costs related to issued convertible bond | - | (2.561) |
| Interests on loans | (2.266) | (382) |
| Interests on leases | (234) | - |
| Proceeds of equity and warrants | 674 | 547 |
| Net cash flows generated from (used in) financing activities | (2.380) | 60.199 |
| Increase (decrease) of cash | 2.116 | 75.092 |
| Exchange rate effects | (650) | (2.544) |
| Cash and cash equivalents at 1 January | 205.159 | 74.348 |
| Total cash and cash equivalents at 31 March | 206.625 | 146.896 |



Outlook for full year 2021

For the remainder of 2021, the Company expects:

- Returning to growth of revenues from sales of RUCONEST[®], mainly driven by the US and expanded EU operations, subject to the progression of the COVID-19 pandemic and quarterly fluctuations in revenues 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[®] and production of leniolisib.
- Investment in pre-marketing activities for leniolisib and the continuing registration-enabling study for leniolisib for APDS, as well as our ongoing clinical trials for rhC1INH and other development activities.
- Continued close monitoring of the ongoing COVID-19 pandemic and the potential impact on the business.

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

No further specific financial guidance for 2021 is provided.

Tickers:

- Euronext Amsterdam: PHARM
- Nasdaq: PHAR

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