Pharming Group N.V.

Half Year Results 2021

05 August 2021

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Company overview

- A revenue generating and profitable dual- listed Dutch bio-pharmaceutical company developing innovative products for the safe, effective treatment of rare diseases and unmet medical needs, established in 1988 with 250+ employees globally
- Investing in development of recombinant human proteins, from own platform, focused on rare and unmet medical need diseases
  - Recombinant human C1-esterase inhibitor (rhC1INH)
    - Enzyme replacement therapy: Lead product marketed as RUCONEST® and approved for the treatment of acute angioedema attacks in patients with hereditary angioedema (HAE)
    - Additional indications in clinical development
- Investing in extension of in-licensed development pipeline
  - Late-stage; leniolisib, for the treatment of activated phosphoinositide 3-kinase delta syndrome (APDS), an ultra rare genetic disease
  - Early-stage; OTL-105, an *ex vivo* HSC gene therapy for HAE
- Profitable from revenues from own commercial infrastructure in the US and EU, and partnerships in Israel, Latin America, Korea, Middle East and North Africa

Operational Highlights: H1 2021

- Reimbursement of RUCONEST® agreed with the Spanish Ministry of Health for the treatment of acute hereditary angioedema (HAE) attacks in Spain.
- Announced the successful completion of patient enrollment in the pivotal Phase 2/3 triple-blind, randomized, placebo-controlled study of leniolisib for the treatment of activated phosphoinositide 3-kinase delta (PI3Kδ) syndrome (APDS). The anticipated launch of leniolisib is in Q4 2022, subject to regulatory approval.
- Announced the launch of navigateAPDS, a sponsored genetic testing program in collaboration with Invitae Corporation (NYSE: NVTA) designed to assist clinicians in identifying patients and their family members with activated PI3K delta syndrome (APDS), which may lead to earlier diagnosis.
- The first patient was enrolled in a Phase IIb double-blind, randomized, controlled study to assess the efficacy of RUCONEST®, for the prevention of acute kidney injury after non-ST elevation myocardial infarction at the University Hospital Basel, Switzerland.
- Upon nomination by the Board of Directors, the Company's Annual General Meeting of Shareholders that was held on 19 May 2021 appointed Steven Baert, Leon Kruimer and Jabine van der Meijs as Non-Executive Directors to the Board.
- Appointed Anurag Relan as Chief Medical Officer and Robert Friesen as Chief Scientific Officer.

Post-period operational highlights

- Entered into an exclusive license agreement with NewBridge Pharmaceuticals for the distribution of RUCONEST® in the Middle East and North Africa.
- Announced a strategic collaboration with Orchard Therapeutics, a global gene therapy leader, to research, develop, manufacture and commercialize OTL-105, a newly disclosed investigational ex-vivo autologous hematopoietic stem cell (HSC) gene therapy for the treatment of HAE. OTL-105 is designed to increase C1 esterase inhibitor (C1INH) in HAE patient serum to prevent HAE attacks.

Three-pillar strategy for growth

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Continuing to grow RUCONEST® sales through further country launches & extending HAE portfolio

- Fully commercialize RUCONEST® in all major international markets with our own sales forces
- Development of early-stage asset, OTL-105, an *ex vivo* HSC gene therapy for HAE gene therapy

  (in-licensed from Orchard Therapeutics)

Grow and extend our HAE franchise

Expanding indications for rhC1INH & developing new recombinant proteins using transgenic technology

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

Extend rhC1INH franchise to larger indications and develop new enzyme replacement therapies

In-licencing or acquisition of drug candidates to meet rare and unmet needs & leverage commercial infrastructure

- In-licensing of late-stage asset, leniolisib, for the treatment of APDS
- In- licensing/ acquisition of additional late- stage assets in rare or ultra- rare diseases

Expand portfolio and leverage commercial infrastructures to grow business

Commercial products and pipeline

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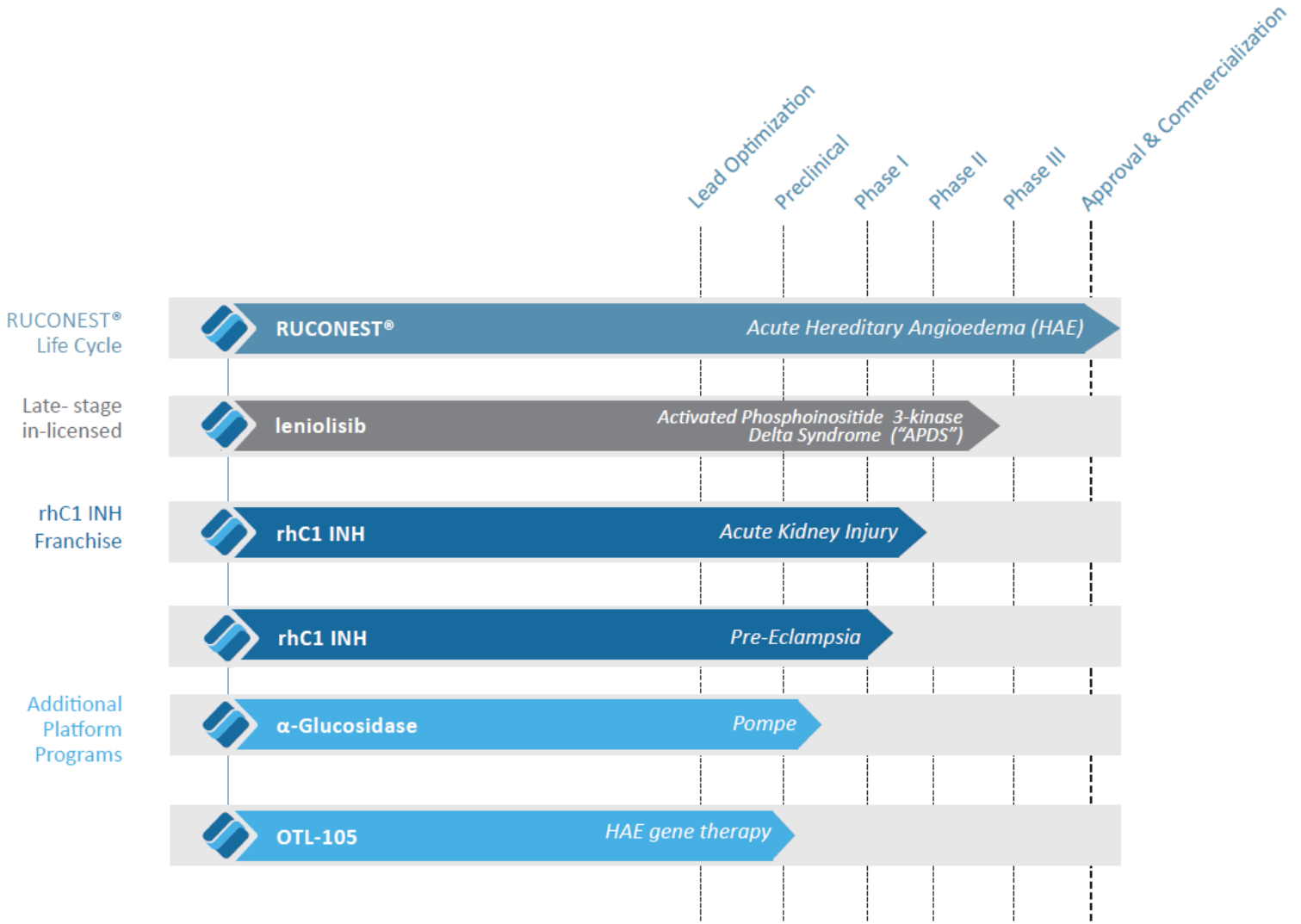
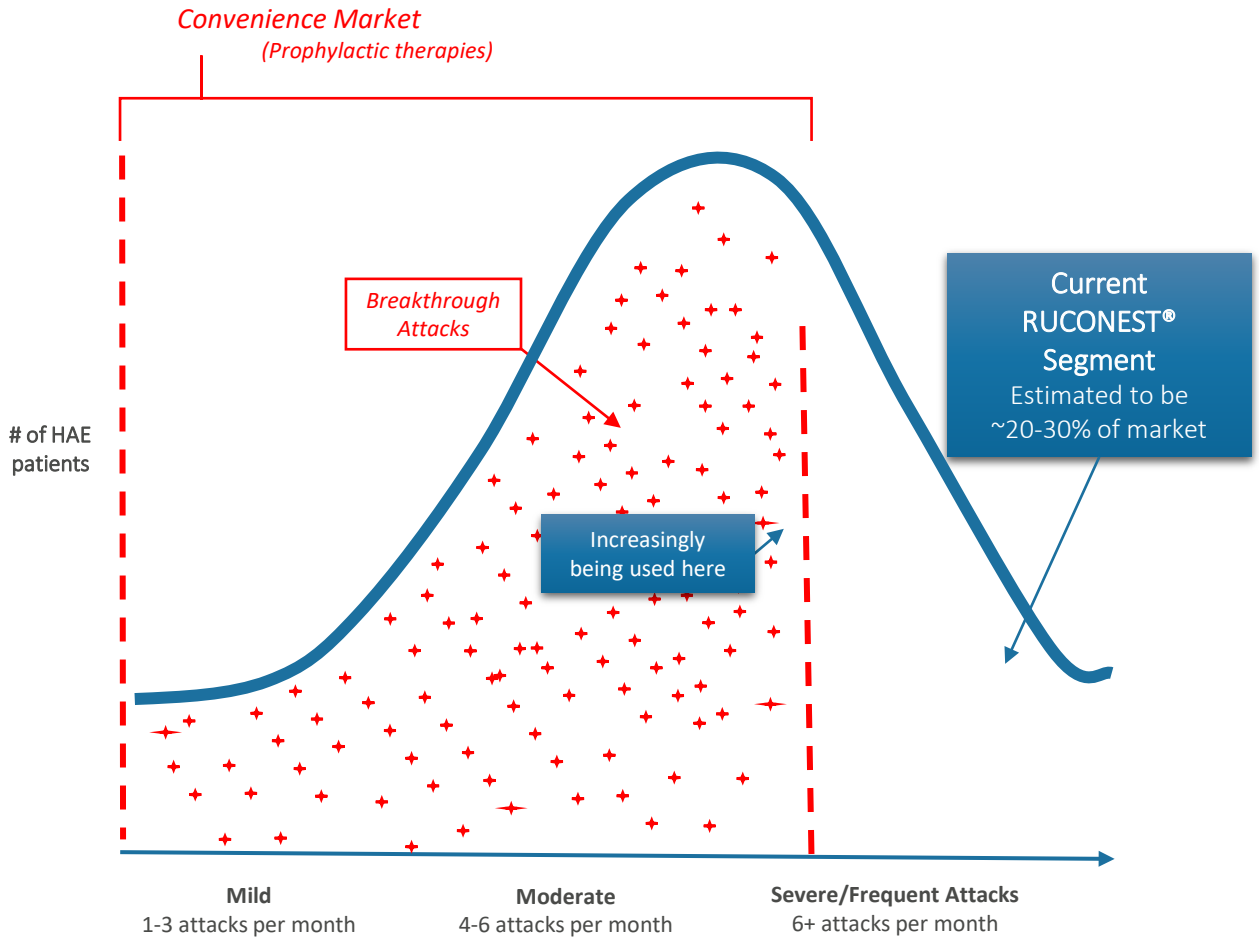


RUCONEST®: enzyme replacement therapy approved for HAE

- HAE is caused by a deficiency of C1-INH, resulting in attacks of severe swelling (angioedema) in various parts of the body

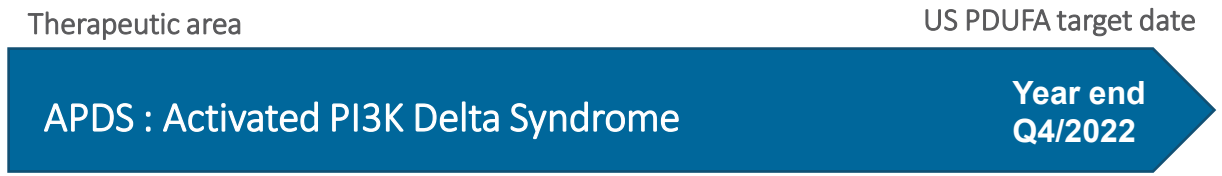
- Patients use medication for treatment and prevention (prophylaxis) of attacks
  - RUCONEST® approved for the treatment of acute HAE attacks in adults and adolescents in the US and EU
  - In 2020, combined sales of therapies totalled more than US$2 billion

- Increasing use of prophylaxis because patients want to be attack-free
  - New treatments offer better attack reduction rates than previous IV plasma-derived C1-INH prophylaxis treatment
  - Although kallikrein/bradykinin inhibitors block the main pathway for symptomatology, C1-INH levels remain low
  - Approx. half of patients using new prophylaxis treatments continue to have breakthrough attacks, some frequently, and are in need of regular use of breakthrough medication



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

Leniolisib in development for the treatment of APDS



Leniolisib[1,2]

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 test[3]

Orphan drug designation granted by US FDA and European Commission

Able to leverage Pharming's existing commercial infrastructure

APDS is ultra-rare primary immunodeficiency (PID)

- Caused by autosomal dominant variations in one of two genes, leading to APDS1 or APDS2
- Results in hyperactivation of phosphoinositide-3-kinase δ (PI3Kδ) which suppresses and dysregulates the immune system
- Balanced PI3Kδ signaling is essential for normal immune function[4,5]

1. Rao VK, et al. Blood. 2017;130(21):2307-2316. 2. Hoegenauer K, et al. ACS Med Chem Lett. 2017;8(9):975-980. 3. Chinn IK, et al. J Allergy Clin Immunol. 2020;145(1):46-69. 4. Okkenhaug K, Vanhaesebroeck B. Nat Rev Immunol. 2003;3:317-330. 5. Fruman DA, et al. Cell. 2017;170(4):605-635.

Leniolisib: potential to address unmet needs in APDS

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Burden of APDS[1-4]

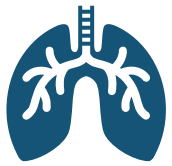
- Estimated >1,350 patients (500 US, 675 EU, 190 Japan) live with APDS (based on prevalence of 1-2/million)
  - Greater understanding of PID's is revealing a larger patient population[5]
- APDS patients are characterized across all global regions
- Years spent undiagnosed or misdiagnosed, seeing 4-5 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 APDS[6]

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

Often used together

- Hematopoietic stem cell transplantation
- No approved therapy for treatment



Severe infections, **permanent lung damage**

GI disease so severe it impairs growth

Severe swollen lymph nodes, spleen and liver

Autoimmunity including severe anemias & bleeding disorders

12-25% of patients succumb to **fatal** lymphoma

1. DOF, Pharming Healthcare, Inc. 2021. 2. Jamee M, et al. Clin Rev Allergy Immunol. 2019;May 21. 3. Maccari ME, et al. Front Immunol. 2018;9:543. 4. Carpier JM, Lucas CL. Front Immunol. 2018;8:2005. 5. Chan AY, et al. *Front Immunol*. 2020;11:239.; IUIS: International Union of Immunological Societies. 6. Coulter TI, Cant AJ. Front Immunol. 2018;9:2043.

Uncovering “APDS” : US Targeted Patient Identification Strategy

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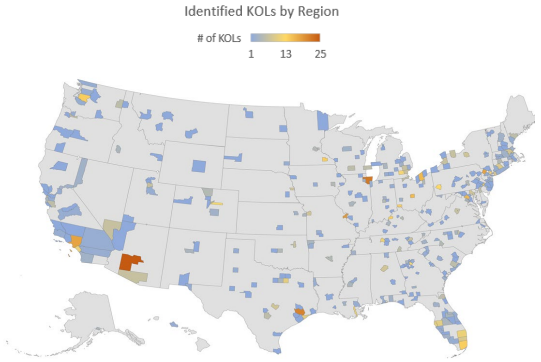
1.

BUILD APDS NETWORK



The US has created a KOL network & referral pathway of prescribers actively supported by field medical & diagnostic liaisons

2.

ANALYTICS & AI

Patient identification using sophisticated & targeted digital strategy & A.I

3.

OUTREACH & EDUCATION

Genetic Testing
navigateAPDS
by Pharming

Immune Deficiency Foundation
Global Genes
Jeffrey Modell Foundation

“Free of charge”genetic testing, supported by strong community connections and social media advocacy

rhC1INH: pipeline in large unmet indications

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- Ongoing Phase IIb clinical trial for rhC1INH in acute kidney injury (AKI) after myocardial infarction
  - Double-blind, randomized controlled study in up to 220 patients at University Hospital of Basel, Switzerland
  - AKI affects approximately 20% of all patients admitted to hospital, incidence may increase to >50% in seriously ill patients or in the presence of risk factors
- Clinical trial for rhC1INH in pre-eclampsia (PE) temporarily halted due to Covid-19
  - Part one: an open label study to investigate tolerability and safety of treatment with rhC1INH
  - Part two: an open label, proof-of-concept study in 30 patients across two centres (Netherlands and Australia)
  - Premature delivery is presently the only solution for PE, and there are no approved therapies
- Ongoing clinical trials for rhC1INH in patients hospitalized with confirmed SARS-CoV-2 infections
  - Multinational, randomized, controlled, investigator-initiated study of up to 150 patients across Switzerland, in Brazil and Mexico
  - Randomized, open-label, parallel-group, controlled, clinical trial in up to 120 participants across centers in the US

OTL-105: developing a best-in-class HAE gene therapy

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- Collaboration with Orchard Therapeutics to develop and commercialize an *ex vivo* autologous hematopoietic stem cell (HSC) gene therapy for HAE
- OTL-105 inserts one or more functional copies of the SERPING1 gene into patients own HSCs *ex vivo* which are then transplanted back into the patient for potential durable C1-INH production
- In preclinical studies, to date, OTL-105 demonstrated high levels of SERPING1 gene expression via lentiviral-mediated transduction in multiple cell lines and primary human CD34+ HSCs. The program also achieved production of functional C1-INH, as measured by a clinically validated assay

Orchard therapeutics™

- Expertise in HSC gene therapy
- Vector development and testing
- Established CDMO network
- Murine transplant studies
- Internal discovery capabilities

+

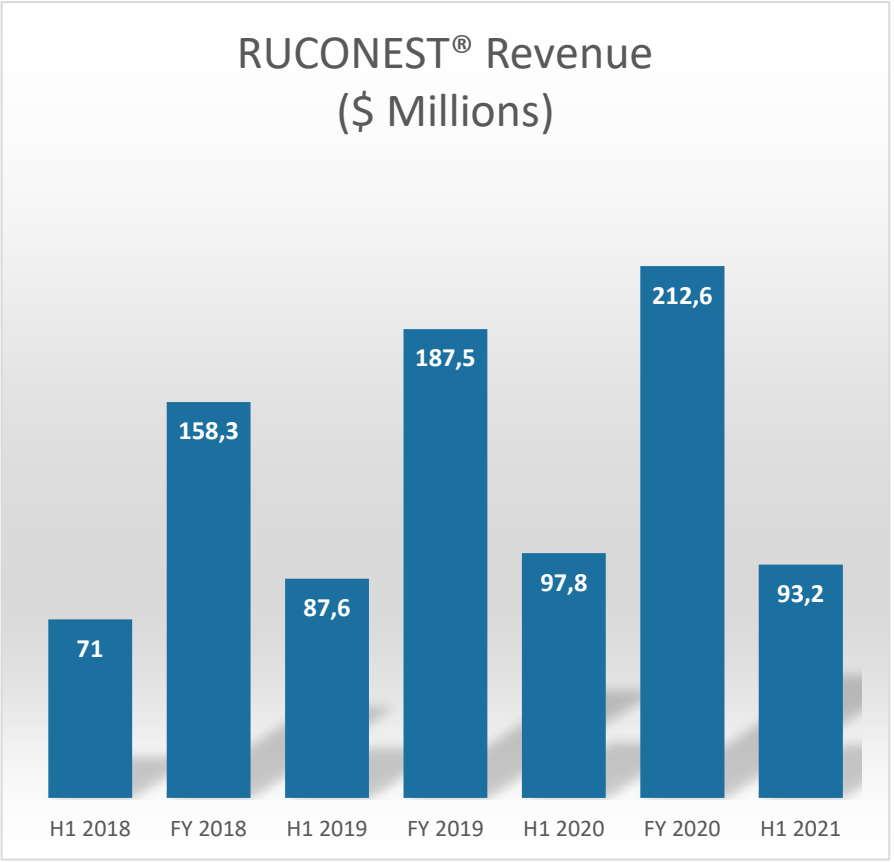
Pharming®

- Extensive clinical and commercial expertise in HAE
- Pre-clinical disease models for HAE
- Capital to fund ongoing development and future commercialisation

Combined expertise and experience to develop a best-in-class HAE gene therapy to provide the potential for life-long prophylaxis following a single administration

Financial Highlights: H1 2021

- As previously noted, Q1 2021 saw the US healthcare economy significantly affected by the second wave of COVID 19 to hit the US.
- In Q2 2021 doctors' offices reopened and diagnostic and routine patient appointments initiated a recovery across the pharmaceutical sector and for RUCONEST sales. The start of the RUCONEST® recovery in the US during Q2 2021 was driven by an increase in new patients and product demand.
- Total revenues for the first half of 2021 came to US$93.2 million (H1 2020: US$97.8 million).
    - Revenues in Q2 2021 increased by 15% to US$49.7 million, compared to US$43.4 million in Q2 2020.
    - Revenues in Q2 2021 also increased by 14% compared to US$43.6 million in Q1 2021.
- For H1 2021 revenue from US sales amounted to US$90.1 million (H1 2020: US$93.9 million).
    - US sales revenues in Q2 2021 increased by 16% to US$48.4 million, (Q1 2021: US$41.6 million).
- Sales revenues in Europe and Rest of World (RoW) totaled US$3.2 million in H1 2021 (H1 2020: US$4.0 million).
    - In Q2 2021 revenue from Europe and RoW sales was US$1.2 million a decrease of 36% on Q1 2021 $1.96 million, mainly as result of phasing of ordering.



Financial Highlights: H1 2021 (2)

- Gross profit for H1 2021 amounted to US$83.8 million compared to H1 2020 (US$86.9 million).
    - Gross profit for Q2 2021 increased by 17% to US$45.0 million compared to US$38.4 million in Q2 2020 and by 16% compared to Q1 2021 in line with the increased revenues.
- Operating profit for the first half of 2021 amounted to US$17.2 million, a 52% decrease from H1 2020: US$35.7 million.
    - Operating profit for Q2 2021 decreased by 23% to US$10.9 million compared to Q2 2020 that totaled US$14.2 million.
    - Other operating costs increased to US$68.0 million compared to US$51.8 million in the first half year of 2020.
    - The increase was a combination of increased R&D expenditure, launch preparation and manufacturing cost for leniolisib, an increase in employee numbers supporting company growth, a significant increase in cost of insurances, an increase in share-based compensation and increased compliance and control costs.
- Net profit for H1 2021 came to US$14.4 million in comparison to H1 2020 (US$20.3 million), as result of lower operating profit offset by currency results and lower funding costs.
- Cash and cash equivalents, together with restricted cash amounted to US$189.8 million at the end of Q2 2021 in comparison to US$206.7 million at the end of 2020 as result of positive cash flow from operating activities (US$16.4 million) reduced by investments the payment of the final $25 million milestone payment in Q2 2021 to Bausch Health Inc. relating to the re-acquisition of the North American RUCONEST® commercialization rights in 2016.

Income statement

Amounts in $ '000	**H1 2021**	**H1 2020**
Revenues	**93,237**	**97,827**
Costs of sales	**(9,487)**	**(10,885)**
Gross profit	**83,750**	**86,942**
Other income	**1,354**	**525**
Research and development	(24,206)	(17,658)
General and administrative	(15,060)	(9,846)
Marketing and sales	(28,686)	(24,283)
Other Operating Costs	**(67,952)**	**(51,787)**
Operating profit	**17,152**	**35,680**
Fair value gain (loss) on revaluation derivatives	44	93
Other finance income	5,398	1,237
Other finance expenses	(2,958)	(8,252)
Finance cost, net	**2,484**	**(6,922)**
Share of net profits in associates using the equity method	**388**	**134**
Profit before tax	**20,024**	**28,892**
Income tax credit (expense)	(5,672)	(8,561)
Profit for the year	**14,352**	**20,331**
Basic earnings per share (€)	0.022	0.032
Diluted earnings per share (€)	0.019	0.028

Balance sheet – assets

Amounts in $ '000	**June 30, 2021**	**31 December 2020**
Non-current assets		
Intangible assets	91,386	94,083
Property, plant and equipment	15,588	12,226
Right-of-use assets	22,043	9,427
Deferred tax assets	23,925	31,877
Investment accounted for using the equity method	7,261	7,118
Restricted cash	493	510
Total non-current assets	**160,696**	**155,241**
Current assets		
Inventories	24,307	21,157
Trade and other receivables	37,550	35,902
Restricted cash	987	995
Cash and cash equivalents	188,303	205,159
Total current assets	**251,147**	**263,213**
Total assets	**411,843**	**418,453**

Balance sheet – liabilities

Amounts in $ '000	**June 30, 2021**	**31 December 2020**
Equity		
Share capital	7,251	7,163
Share premium	453,014	444,940
Legal reserves	14,665	19,859
Accumulated deficit	(276,858)	(288,527)
Shareholders' equity	**198,072**	**183,435**
Non-current liabilities		
Convertible bonds	145,437	149,727
Lease liabilities	20,328	8,230
Other financial liabilities	189	212
Total non-current liabilities	**165,954**	**158,169**
Current liabilities		
Convertible bonds	1,972	2,040
Derivative financial liabilities	71	181
Trade and other payables	43,123	47,666
Lease liabilities	2,651	1,962
Other financial liabilities	—	25,000
Total current liabilities	**47,817**	**76,849**
Total equity and liabilities	**411,843**	**418,453**

Cash flow

Amounts in $'000	**H1 2021**	**H1 2020**
Profit before tax	**20,024**	**28,892**
Net cash flows generated from (used in) operating activities	**16,418**	**44,875**
Capital expenditure for property, plant and equipment	(5,436)	(1,143)
Investment intangible assets	(1,206)	(254)
Investment associate	-	(14)
Acquisition of license	(1,083)	(8,767)
Net cash flows used in investing activities	**(7,725)**	**(10,178)**
Repayment on loans and borrowings	—	(55,117)
Payment on contingent consideration	(25,000)	(20,025)
Payment of lease liabilities	(1,618)	(1,548)
Proceeds of issued convertible bond	—	135,470
Interests on loans	(2,261)	(795)
Proceeds of equity and warrants	3,867	2,116
Net cash flows generated from (used in) financing activities	**(25,012)**	**60,101**
Increase (decrease) of cash	**(16,319)**	**94,798**
Exchange rate effects	(537)	2,062
Cash and cash equivalents at 1 January	205,159	74,348
Total cash and cash equivalents at 30 June	**188,303**	**171,208**

Investment summary

- A well funded business supported by commercial sales and a growing pipeline for the treatment of rare diseases and unmet medical needs
- Lead product from platform, RUCONEST® (rhC1INH), launched in over 40 countries with sales of over US$93m in H1 2021
- Potential near-term inflection point with anticipated end of 2022 launch of leniolisib, in-licensed from Novartis, for the treatment of orphan disease APDS
- Targeting new, large indications for rhC1INH with Phase II studies
- Earlier-stage pipeline assets include in-licensed curative gene therapy treatment for HAE and own transgenic platform-derived candidate for Pompe disease
- Able to leverage established commercial infrastructure across US and Europe for in-licensed products and expanding manufacturing capacity to support continued RUCONEST® demand and rhC1INH pipeline
- Experienced leadership team and strong balance sheet to support ambitious growth strategy, including potential M&A

Outlook for remainder of 2021

For the remainder of 2021, we expect:

- Continued increase in revenues from the sales of RUCONEST®, as a result of the pharmaceutical market continuing to normalize and return to its pre-COVID 19 state. We will though continue to monitor the situation in all markets and continue to expect some periodic disruptions.
- Maintenance of positive net earnings during the year.
- Investments in acquisitions and in-licensing of new development opportunities and assets.
- Continued investment in the expansion of production facilities, both for RUCONEST® and leniolisib.
- Investment in launch-critical medical affairs and pre-marketing activities for leniolisib and the registration-enabling study for APDS, as well as our ongoing clinical trials for rhC1INH and other development activities, including OTL-105.

No further specific financial guidance for 2021 is provided.

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