Pharming Group N.V.

9M 2021 Financial Results
Analyst Call

28 October 2021

NASDAQ: **PHAR** | Euronext Amsterdam: **PHARM**

Forward looking statements

This presentation may contain forward-looking statements. Forward-looking statements are neither historical facts nor assurances of future performance. Instead, they are based on our current beliefs, expectations and assumptions regarding the future of our business, future plans and strategies, our development plans, our clinical results and other future conditions. All statements other than statements of historical facts contained in this presentation, including statements regarding our future financial or business performance, conditions, plans, prospects, trends or strategies, objectives of management and other financial and business matters; our current and prospective product candidates, planned clinical trials and preclinical studies, projected research and development costs, current and prospective collaborations; and the estimated size of the market for our product candidates, the timing and success of our development and commercialization of our product candidates and the market acceptance thereof, are forward-looking statements. New risks and uncertainties may emerge from time to time, and it is not possible to predict all risks and uncertainties. While we may elect to update these forward-looking statements at some point in the future, we assume no obligation to update or revise any forward-looking statements except to the extent required by applicable law. Although we believe the expectations reflected in such forward-looking statements are reasonable, we can give no assurance that such expectations will prove to be correct. Accordingly, readers are cautioned not to place undue reliance on these forward-looking statements. No representations or warranties (expressed or implied) are made about the accuracy of any such forward-looking statements.

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Agenda

Introduction & Operational Growth – Sijmen de Vries, CEO

Medical & Clinical Update – Anurag Relan, CMO

Financial Review – Jeroen Wakkerman, CFO

Q&A

Execution delivering future growth

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- 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$146 in 9M 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

Three-pillar strategy to build a fully integrated sustainable business

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Grow and extend our HAE franchise

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)

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

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

Expand portfolio and leverage commercial infrastructures to grow business

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

Strategy in action: Progress during 9M 2021

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Commitment to HAE

- In-licensing of OTL-105 from Orchard Therapeutics for a potential cure for HAE
- Expanding reach of RUCONEST® with commercialization agreement with NewBridge Pharmaceuticals in North Africa and the Middle East
- RUCONEST® reimbursement in Spain

Expanding indications

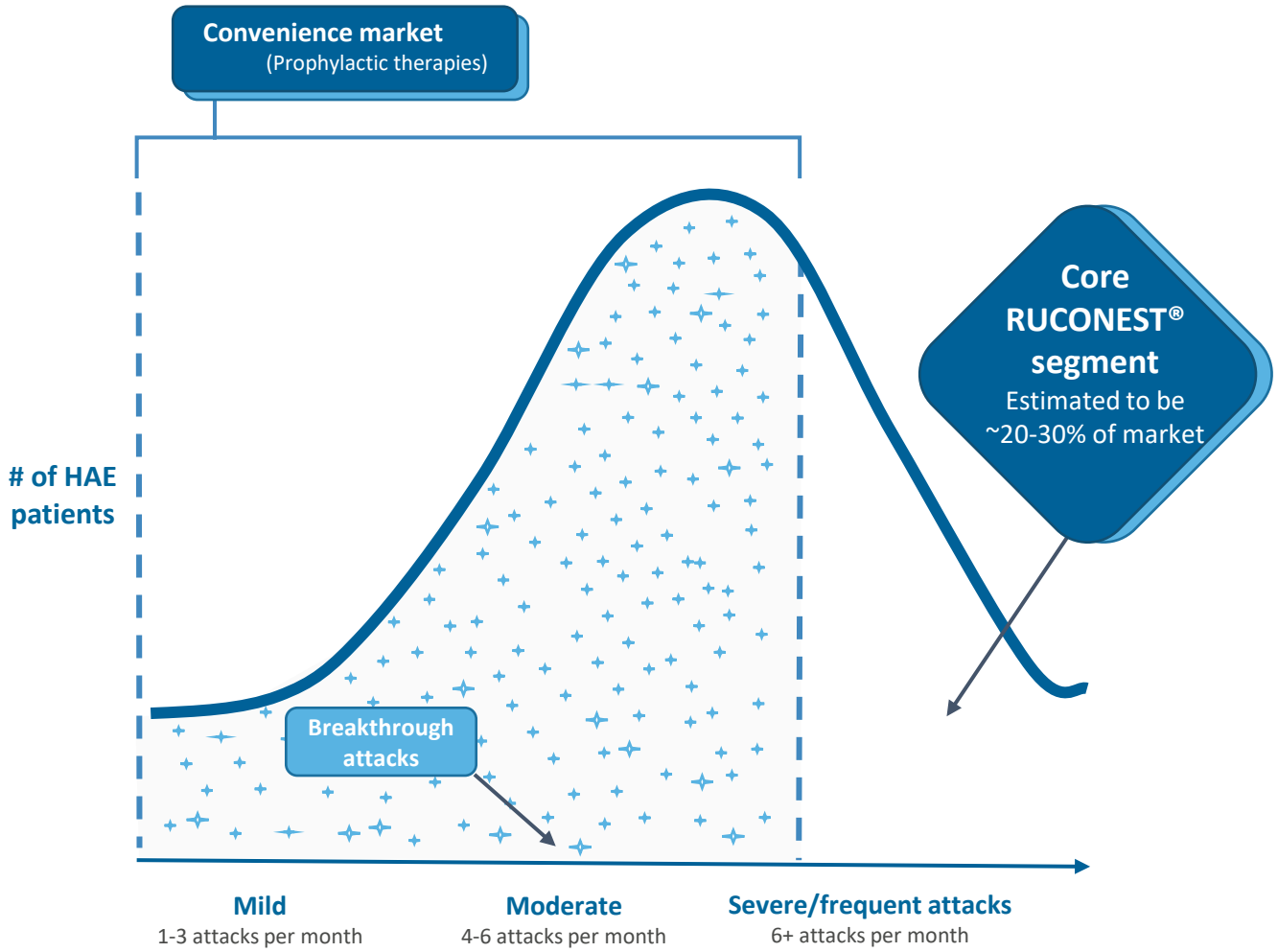
- Acute Kidney Injury trial restarted following COVID-19 delay
- Topline results from rhC1INH clinical trial in severe pneumonia as a result of COVID-19 infection

In-licensing and acquisitions

- Completion of enrolment in Phase II/III study with leniolisib for activated PI3Kδ syndrome
- Investments in pre-launch activities for anticipated Q4 2022 regulatory approval of leniolisib

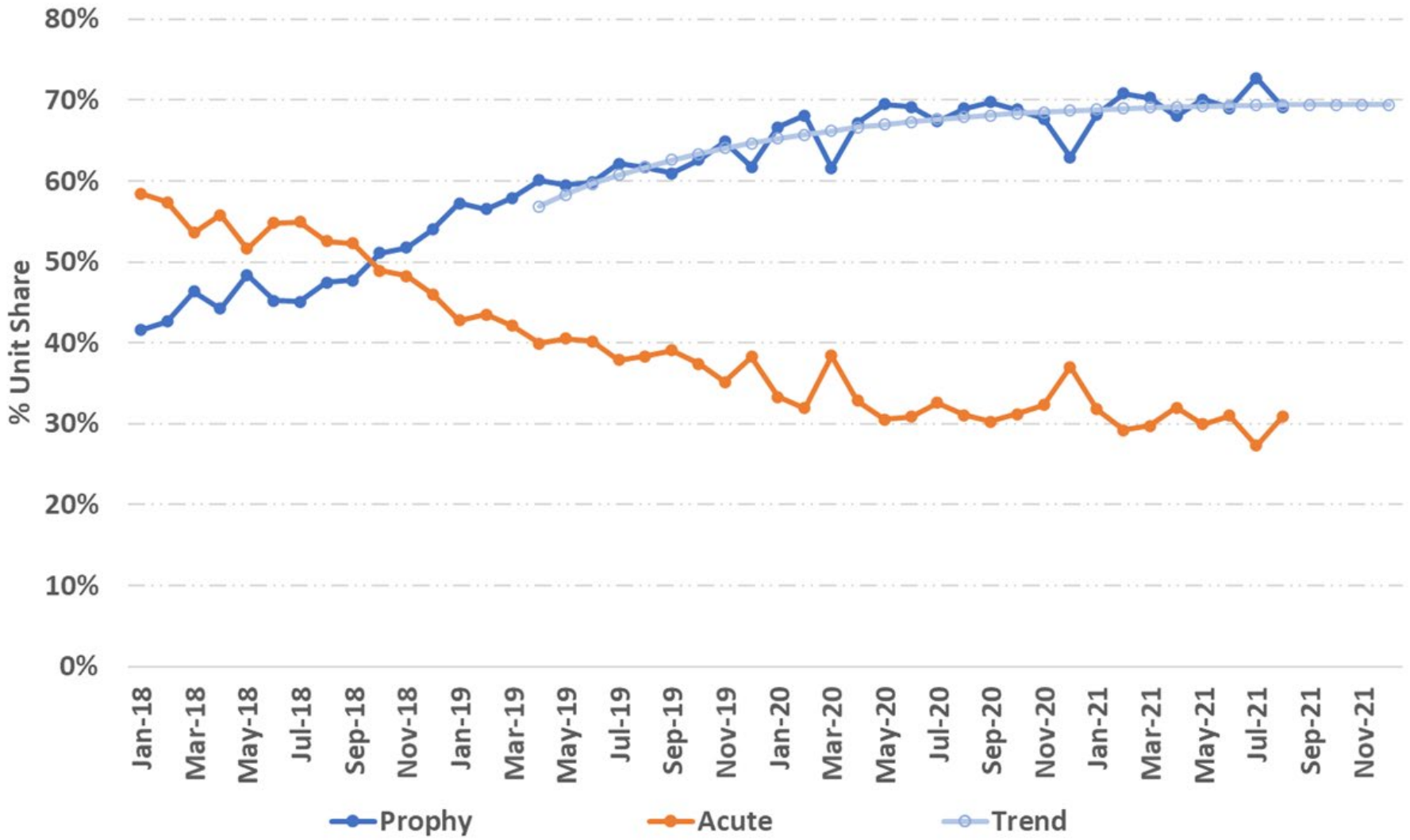
RUCONEST® positioning in the treatment of HAE

- 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 regularly use of breakthrough medication



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

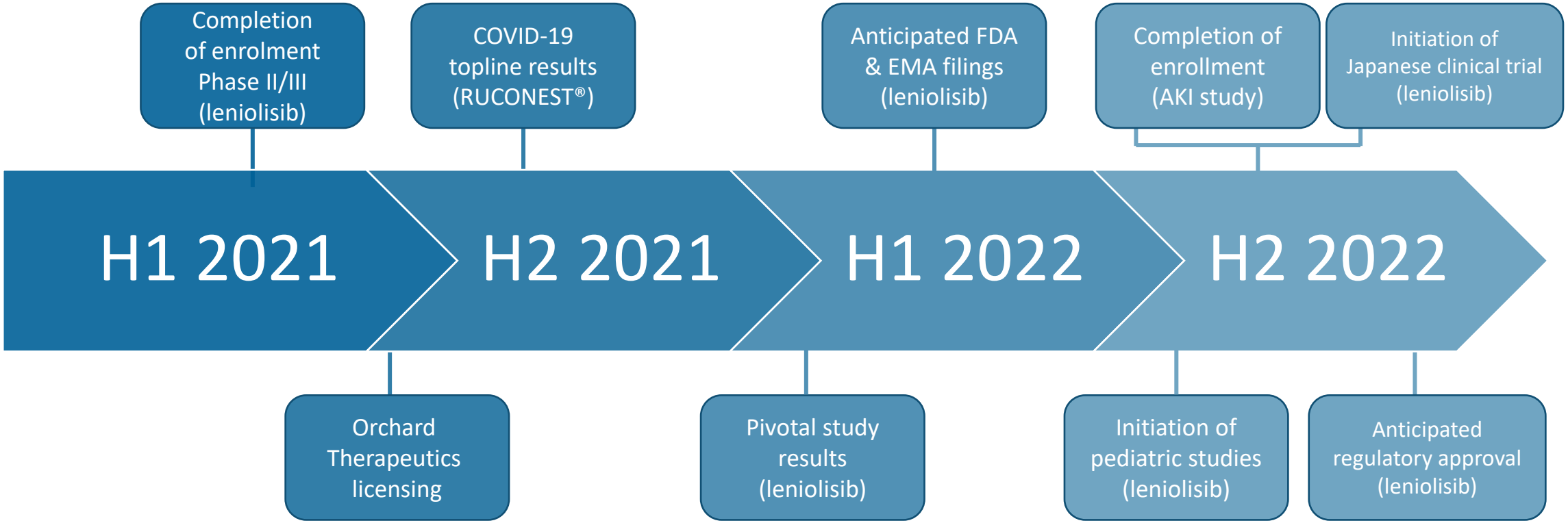
Ongoing demand for acute therapy following stabilization of prophylactic market



Data up to and including August 31, 2021

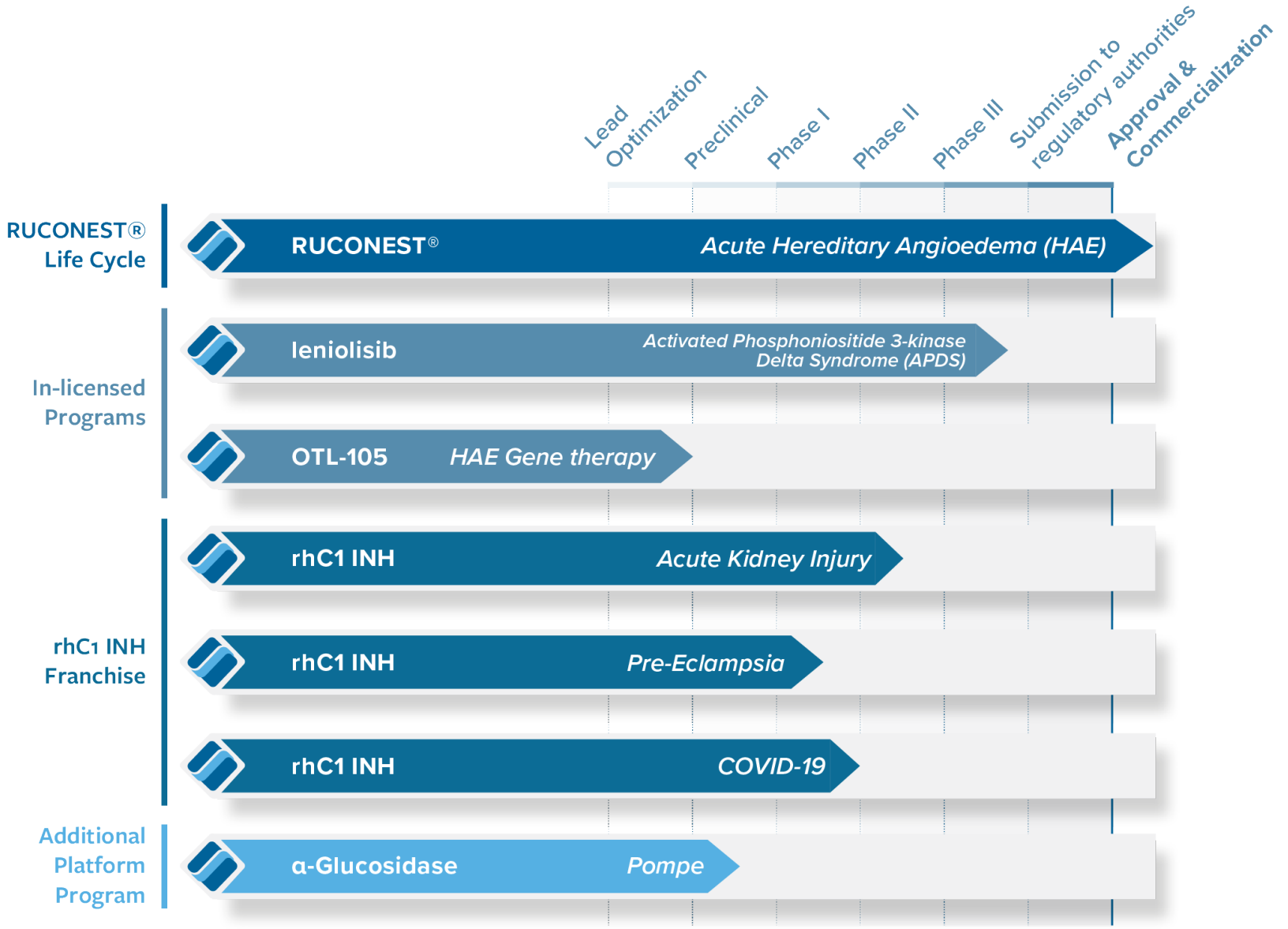
Based on partial payer data, that we estimate represents ~25% of the market

Key near-term value inflection points as we build a sustainable business*



These dates are not an assurance of future performance; they are based on current expectations and assumptions regarding the future of our business. Please refer to our Forward-looking Statement on slide 2 of this presentation.

Commercial products and focused pipeline to support long-term growth



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HAE & OTL-105

Grow and extend our HAE franchise

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

- 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

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- 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

The HSC gene therapy approach

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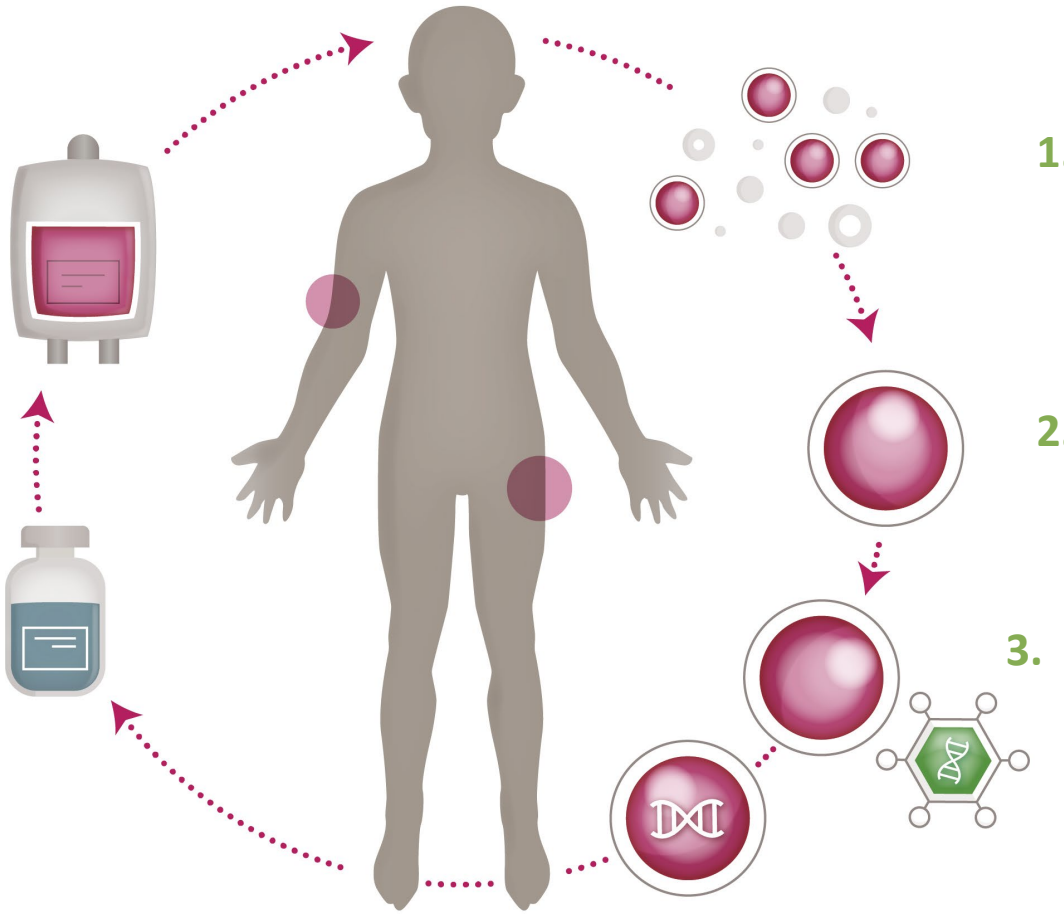
1. **Cells are collected from the patient**

2. **Blood stem cells are selected and purified**

3. **A working copy of the gene is inserted into the cells using a viral vector**
   Gene-corrected cells are frozen, or cryopreserved

4. **The patient is conditioned to receive treatment**

5. **The gene-corrected cells are infused into the patient**



HSC gene therapy has led to multiple approved and effective products

Modality	HSC Gene Therapy	AAV- GT	Gene Editing
Proven Approach	✓ – Multiple products approved and pipeline with impressive data – HSC GT and CAR-T drive further innovation	✗ – No liver-directed AAV is approved – Selectivity for specific cells has proven difficult	✗ – No approved products
Efficacy	✓ – Based on other clinical programs, expression levels appear achievable	? – High amount of protein has proven to be very challenging for AAV – Antibodies to AAV	? – Unsure, pre-clinical data appears promising – Rationale based on lanadelumab
Durability of Effect	✓ – Durability of effect has been proven in other programs	? – Decreased expression levels observed Hemophilia A	✓ – Theoretically, should be permanent
Safety	✓ – Autologous HSCT is approved and appears safe	? – Immune responses to target cells – Significant questions remain	? – Promising but no conclusions can be made – No off-switch on kallikrein inhibition

APDS & leniolisib

Expanding our portfolio and leveraging our commercial infrastructure to grow our business

Significant unmet need in APDS (Activated PI3Kδ Syndrome)

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Burden of APDS[1-4]

- Estimated >1,350 patients (500 US, 675 EU, 190 Japan) live with APDS
  - More than 350 patients already identified by Pharming
  - Greater understanding of PID's is revealing a larger patient population[5]
- 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]

- Supportive/non-specific 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

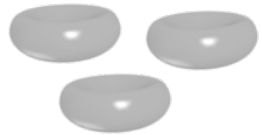
- Hematopoietic stem cell transplantation
- No approved therapy for treatment

Severe infections, permanent lung damage

Severe GI disease

Severe swollen lymph nodes, spleen and liver

Autoimmunity including severe anemias



12-25% of patients succumb to 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. 6. Bousfiha A, et al. J Clin Immunol. 2020;40(1):66-81.

Leniolisib in development for the treatment of APDS

US PDUFA target date

APDS : Activated PI3K Delta Syndrome → **Year end Q4/2022**

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.

Uncovering “APDS”: US targeted patient identification strategy

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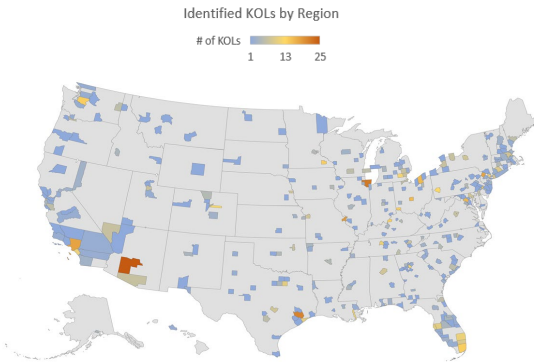
1.

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

Leniolisib: Data on track to readout early 2022 – Pivotal trial design [1,2]

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Part 1
Dose discovery

12 weeks
N=6

Leniolisib
10, 30 and 70 mg BID

- Non-randomized, open-label, dose-escalation study
- Population: Adults with APDS-associated mutation in the PI3Kδ gene (p110δ, i.e., PIK3CD), lymphoproliferation and APDS-typical clinical manifestations/history
- Primary outcomes: Safety & tolerability, PK/PD, pAKT inhibition
- Oral dose 70 mg BID selected for phase III

Part 2 –
Placebo controlled phase III

Randomized period
12 weeks
N=30

Leniolisib 2:1
70 mg BID

Placebo

- Randomized, triple-blinded (patient, caregiver, investigator), placebo-controlled, fixed-dose study
- Co-primary efficacy endpoints (lymphadenopathy and immunophenotype normalization)
  - Change from baseline in the index lesions selected as per from MRI/CT imaging
  - Change from baseline in percentage of naïve B cells out of total B cells
- Enrollment completed

Extension phase
Leniolisib

Leniolisib is an investigational new drug that has not been approved for any use.

1. ClinicalTrials NCT02435173. https://clinicaltrials.gov/ct2/show/NCT02435173 Accessed July 17, 2020. **2.** Rao VK, et al. *Blood.* 2017;130(21):2307–2316.

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Financial highlights from 9M 2021: Building a sustainable business (1/6)

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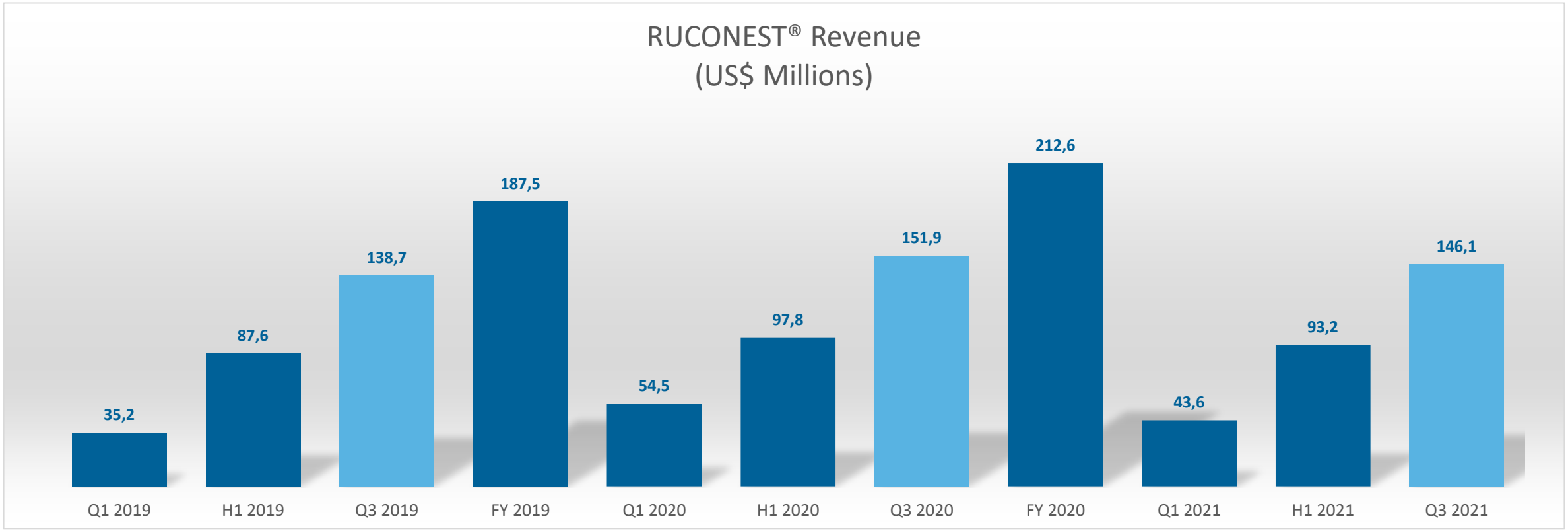
REVENUE

- Revenues in Q3 2021 were US$52.9 million a 6% increase from US$49.7 million in Q2 2021
- Revenues for 9M 2021 were US$146.1 million a 4% decrease compared to 9M 2020 (US$151.9 million).
  - Ongoing recovery in sales following the impact of COVID-19 on the US healthcare economy in Q1 2021, as previously noted in our Q1 2021 and Q2 2021 financial reports.



Financial highlights from 9M 2021: Building a sustainable business (2/6)

Pharming

SALES

- RUCONEST® US sales continue to recover in Q3 2021 with an increase of 6% to US$51.1 compared to Q2 2021 (US$48.4 million).
- US RUCONEST® sales for 9M 2021 were US$141.1 million, a 3% decrease compared to US$145.9 million in 9M 2020.
- In Q3 2021 Europe & Rest of World sales were €1.9 million, an increase of 58% compared to Q2 2021 (US$1.2 million).
- 9M RUCONEST® sales in Europe & RoW were US$5.0 million for 9M 2021 (9M 2020: US$6.1 million).

GROSS PROFIT

- Gross profit increased by 4% in Q3 2021 US$46.9 million compared to Q2 2021 US$45.0 million, in line with the increased quarter-on-quarter revenues.
- Gross profit for 9M 2021 was US$130.6 million a 3% decrease in comparison to 9M 2020 (US$135.3 million).

Financial highlights from 9M 2021: Building a sustainable business (3/6)

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OPERATING PROFIT & COST

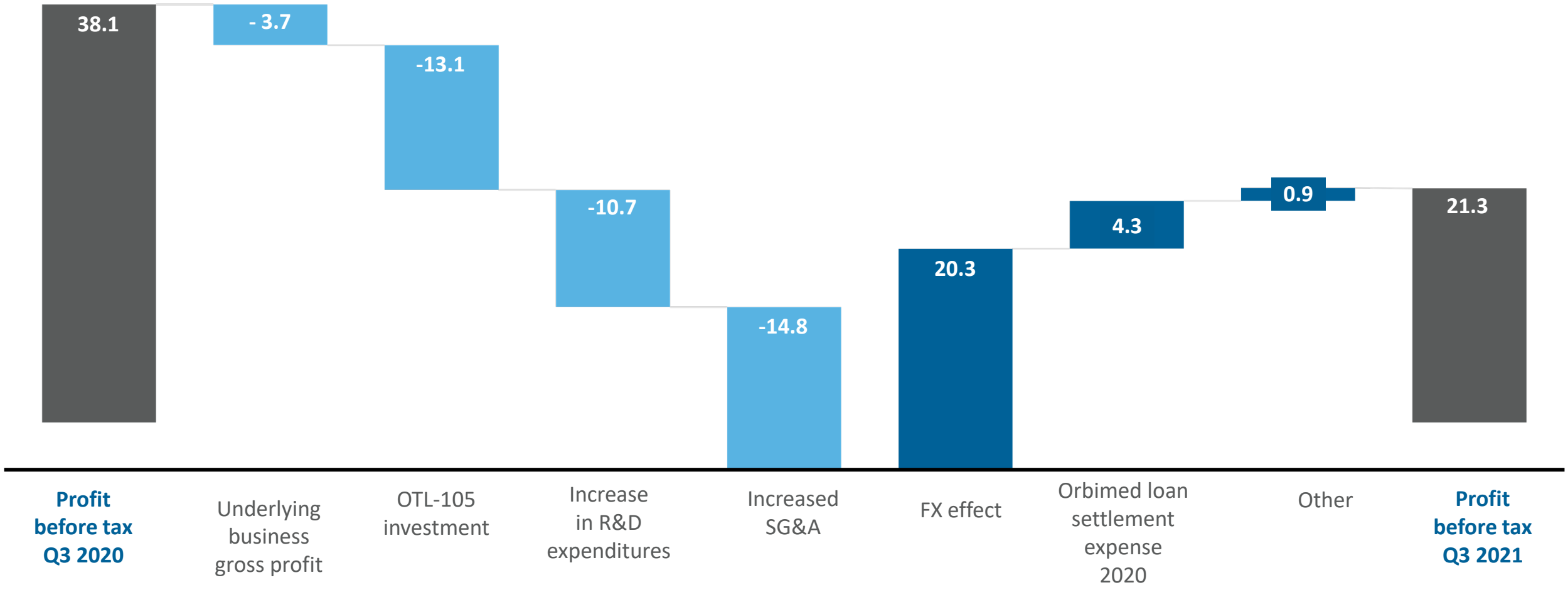
- Operating profit for 9M 2021 was US$15.3 million decreasing 72% on 9M 2020 (US$57.7 million).
  - Due to increased operating cost, including significant investments in the pipeline, OTL-105 license (US$13.1 million), and increased costs of corporate development.
- Operating costs increased to US$116.4 million compared to US$78.5 million in 9M 2020. The increase is due to investment in Pharming's long-term growth:
  - increased R&D expenditure;
  - the cost of the OTL-105 license (US$13.1 million);
  - leniolisib pre-launch marketing preparations and manufacturing cost for leniolisib;
  - an increase in employee numbers to support growth and in share-based compensation;
  - In addition, the increase in insurance, compliance and control costs relating to the recent Nasdaq listing, as previously noted.

NET PROFIT

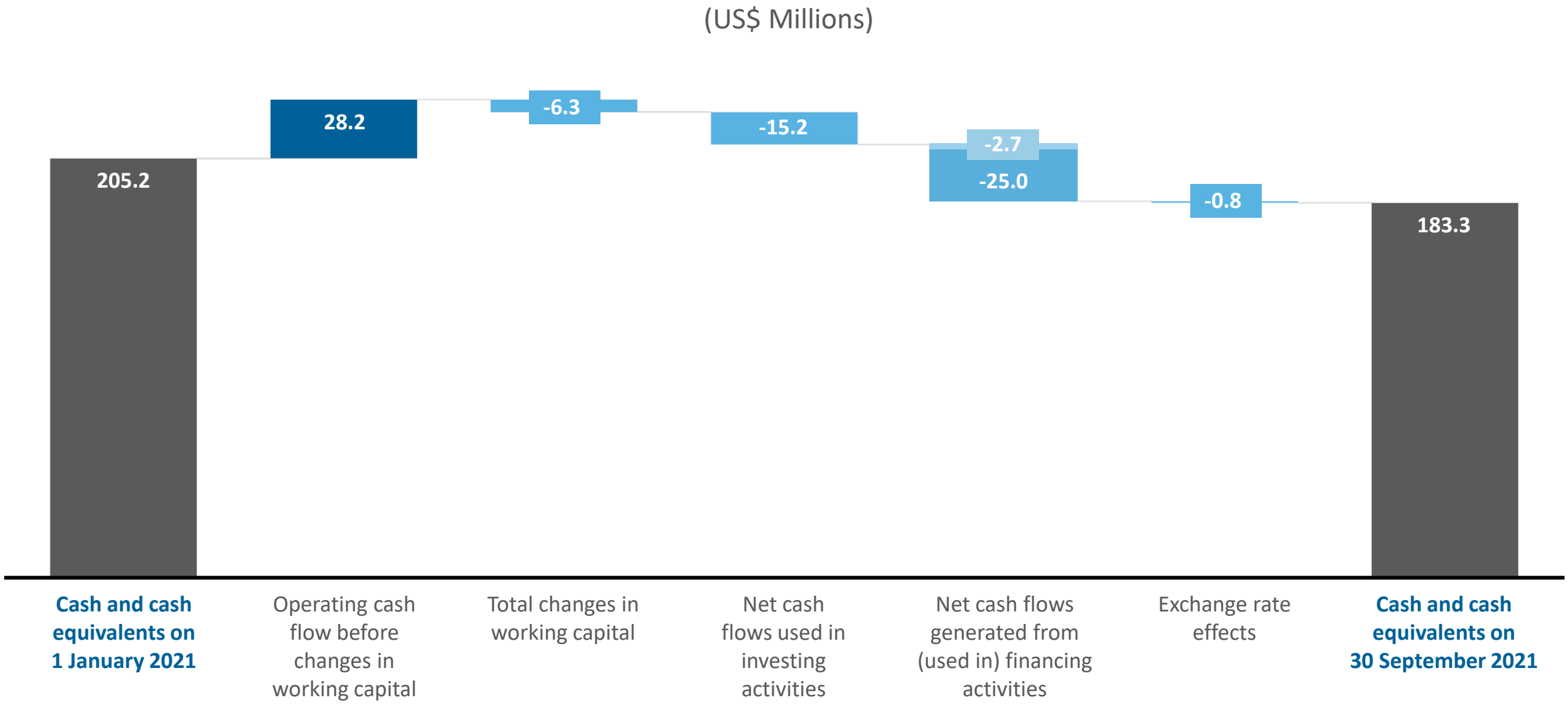
- Net profit for 9M 2021 was US$13.9 million a 49% decrease compared to 9M 2020 (US$28.9 million)
  - As a result of initial in-licensing cost of OTL-105 (US$13.1 million) leading to lower operating profit which was offset by currency exchange rates results and lower funding costs.

Financial highlights from 9M 2021: Profit before tax Q3 2020 – Q3 2021 (4/6)

(US$ Millions)



9M 2020: Cashflow 1 January 2021 – 30 September 2021 (5/6)



Financial highlights from 9M 2021: Building a sustainable business (6/6)

CASH & CASH EQUIVALENTS

- Cash and cash equivalents, together with restricted cash, decreased from US$206.7 million at the end of 2020 to US$184.8 million at the end of Q3 2021.
  - This was as a result of positive cash flows from operating activities of US$21.9 million remaining after the US$13.1 million one- off payment to Orchard Therapeutics and reduced by investments and negative financing cash flows totaling US$42.9 million.
  - The US$42.9 million includes investments in production facilities and the payment of the final US$25.0 million milestone to Bausch Health Inc. in Q2 2021 in relation to the re-acquisition of the North American RUCONEST® commercialization rights in 2016.

Financial outlook

For the remainder of 2021, we continue to expect:

- Continued quarter on quarter increase in revenues from RUCONEST® sales due to normalizing pharmaceutical markets following the impact of COVID-19. However, we will continue to monitor the situation in all markets and could expect some periodic disruptions.
- Maintenance of positive net earnings during the remainder of the year.
- Significant and increasing investment in launch-critical medical affairs and pre-marketing activities for leniolisib as well as continued investment in ongoing clinical trials for rhC1INH and other development activities, including OTL-105.
- Investments in acquisitions and in-licensing of new development opportunities and assets.

No further specific financial guidance for 2021 is provided.

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Q&A

Sijmen de Vries
Chief Executive Officer

Anurag Relan
Chief Medical Officer

Jeroen Wakkerman
Chief Financial Officer

Stephen Toor
Chief Commerical Officer

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NASDAQ: PHAR

Bloomberg: PHAR.AS

This presentation and a recording of this call will be made available on the company's website.

Statement of profit and loss

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Amounts in $ '000	YTD 2021	YTD 2020
Revenues	**146,101**	**151,874**
Costs of sales	**(15,500)**	**(16,566)**
Gross profit	**130,601**	**135,308**
Other income	**1,808**	**810**
Research and development	(37,580)	(26,842)
OTL-105 in-licensing	(13,105)	0
General and administrative	(22,510)	(15,411)
Marketing and sales	(43,880)	(36,204)
Other Operating Costs	**(117,075)**	**(78,457)**
Operating profit	**15,334**	**57,661**
Fair value gain (loss) on revaluation derivatives	59	147
Other finance income	9,907	655
Other finance expenses	(4,466)	(20,614)
Finance cost net	**5,500**	**(19,812)**
Share of net profits in associates using the equity method	**511**	**219**
Profit before tax	**21,345**	**38,068**
Income tax credit (expense)	(7,412)	(9,212)
Profit for the year	**13,933**	**28,856**
Basic earnings per share ($)	0.022	0.045
Fully-diluted earnings per share ($)	0.018	0.039

Balance sheet – assets

Amounts in $ '000	30 September 2021	31 December 2020
Non-current assets		
Intangible assets	89,009	94,083
Property, plant and equipment	16,914	12,226
Right-of-use assets	20,982	9,427
Long-term prepayments	198	0
Deferred tax assets	21,473	31,877
Investments accounted for using the equity method	7,187	7,118
Investment in equity instruments designated as at FVTOCI	2,483	0
Restricted cash	481	510
Total non-current assets	**158,727**	**155,241**
Current assets		
Inventories	25,098	21,157
Trade and other receivables	32,810	35,901
Restricted cash	981	995
Cash and cash equivalents	183,324	205,159
Total current assets	**242,213**	**263,212**
Total assets	**400,940**	**418,453**

Balance sheet – liabilities

Amounts in $ '000	30 September 2021	31 December 2020
Equity		
Share capital	7,259	7,163
Share premium	453,476	444,940
Legal reserves	9,864	19,859
Accumulated deficit	(277,053)	(288,527)
Shareholders' equity	**193,546**	**183,435**
Non-current liabilities		
Convertible bonds	140,962	149,727
Lease liabilities	19,323	8,230
Other financial liabilities	386	212
Total non-current liabilities	**160,671**	**158,169**
Current liabilities		
Convertible bonds	1,923	2,040
Derivative financial liabilities	54	181
Trade and other payables	42,151	47,666
Lease liabilities	2,595	1,962
Other financial liabilities	0	25,000
Total current liabilities	**46,723**	**76,849**
Total equity and liabilities	**400,940**	**418,453**

Cash flow (1/2)

Amounts in $'000	YTD 2021	YTD 2020
Profit before tax	**21,345**	**38,068**
Non-cash adjustments:		
Depreciation, amortization, impairment	6,867	5,741
Equity settled share based payments	5,706	2,365
Fair value gain (loss) on revaluation of derivatives	(59)	(148)
Other finance income	(9,907)	(655)
Other finance expense	4,466	20,616
Share of net profits in associates using the equity method	(511)	(220)
Other	272	2,489
Operating cash flows before changes in working capital	**28,179**	**68,256**
Changes in working capital:		
Inventories	(3,941)	(2,159)
Trade and other receivables	3,092	(799)
Payables and other current liabilities	(5,514)	(1,979)
Restricted Cash	42	1,074
Total changes in working capital	**(6,321)**	**(3,863)**
Interest received	51	655
Income taxes paid	0	(2,741)
Net cash flows generated from (used in) operating activities	**21,909**	**62,307**

Cash flow (2/2)

Amounts in $'000	YTD 2021	YTD 2020
Capital expenditure for property, plant and equipment	(7,451)	(1,551)
Investment intangible assets	(1,544)	(374)
Investment in equity instruments designated as at FVTOCI	(4,589)	0
Acquisition of license	(1,593)	(9,523)
Net cash flows used in investing activities	**(15,177)**	**(11,448)**
Repayment on loans and borrowings	0	(56,273)
Payment on contingent consideration	(25,000)	(20,445)
Payment of lease liabilities	(2,476)	(1,489)
Proceeds of issued convertible bonds	0	138,312
Interests on loans and leases	(4,493)	(3,072)
Proceeds of equity and warrants	4,237	2,294
Net cash flows generated from (used in) financing activities	**(27,732)**	**59,327**
Increase (decrease) of cash	**(21,000)**	**110,816**
Exchange rate effects	(835)	(3,486)
Cash and cash equivalents at 1 January	205,159	74,348
Total cash and cash equivalents at 30 September	**183,324**	**181,048**