Pharming®

Pharming Group N.V.

9M 2022 Financial Results Analyst Call

October 27, 2022

NASDAQ: **PHAR** | EURONEXT Amsterdam: **PHARM**

Forward looking statements

This presentation may contain forward-looking statements. Forward-looking statements are statements of future expectations that are based on management's current expectations and assumptions and involve known and unknown risks and uncertainties that could cause actual results, performance, or events to differ materially from those expressed or implied in these statements. These forward-looking statements are identified by their use of terms and phrases such as "aim", "ambition", ''anticipate'', ''believe'', ''could'', ''estimate'', ''expect'', ''goals'', ''intend'', ''may'', "milestones", ''objectives'', ''outlook'', ''plan'', ''probably'', ''project'', ''risks'', "schedule", ''seek'', ''should'', ''target'', ''will'' and similar terms and phrases. Examples of forward-looking statements may include statements with respect to timing and progress of Pharming's preclinical studies and clinical trials of its product candidates, Pharming's clinical and commercial prospects, and Pharming's expectations regarding its projected working capital requirements and cash resources, which statements are subject to a number of risks, uncertainties and assumptions, including, but not limited to the scope, progress and expansion of Pharming's clinical trials and ramifications for the cost thereof; and clinical, scientific, regulatory and technical developments. In light of these risks and uncertainties, and other risks and uncertainties that are described in Pharming's 2021 Annual Report and the Annual Report on Form 20-F for the year ended December 31, 2021, filed with the U.S. Securities and Exchange Commission, the events and circumstances discussed in such forward-looking statements may not occur, and Pharming's actual results could differ materially and adversely from those anticipated or implied thereby. All forward-looking statements contained in this press release are expressly qualified in their entirety by the cautionary statements contained or referred to in this section. Readers should not place undue reliance on forward-looking statements. Any forward-looking statements speak only as of the date of this presentation and are based on information available to Pharming as of the date of its release. Pharming does not undertake any obligation to publicly update or revise any forward-looking statement as a result of new information, future events or other information.

Pharming®

SPEAKERS

Sijmen de Vries, MD
Chief Executive Officer

Anurag Relan, MD
Chief Medical Officer

Jeroen Wakkerman
Chief Financial Officer

Pharming®

CEO

Sijmen de Vries, MD

Chief Executive Officer

Strategic and operational highlights

Pharming's strategic objectives

- **Building a sustainable business by focusing on RUCONEST® sales**
- **Focus on market approval, launch and commercialization of leniolisib in key markets of US, UK and EEA**
- **Ongoing pipeline development and management of rare disease assets**

Continuing to build a sustainable business in rare diseases

Building a sustainable business by focusing on RUCONEST® sales

Positive cash flow from RUCONEST® helps fund leniolisib and pipeline development and management

- Fully commercialize RUCONEST® in all major international markets with our own sales forces

Anticipated approval and commercialization of leniolisib

Successful commercialization of leniolisib and life cycle management of future indications

- Market opportunity with an estimated >1,350 patients* (500 US, 675 EU, 190 Japan) living with APDS and more than 400 patients already identified by Pharming
- Developing PI3Kδ for additional indications for rare disease patients

* Size based on population and available literature

Ongoing pipeline development and management of rare disease assets

Development through internal projects and potential acquisitions new, late-stage assets through in-licensing and M&A opportunities

- Development of OTL-105, an ex-vivo HSC gene therapy candidate for HAE
- Development of rhaGLU, an enzyme replacement therapy for Pompe disease

Strategic highlights: leniolisib progress (1/2)

USA

SEP 28

Filing and acceptance for Priority Review of New Drug Application to the FDA. Assigned a Prescription Drug User Fee Act (PDUFA) goal date of March 29, 2023

OCT 1

International Classification of Diseases, 10th Revision, Clinical Modification (ICD-10-CM) by the US CDC for APDS took effect

Q1 2023

Remain on track for commercial approval of leniolisib in the first quarter of 2023. Commercialization in the second quarter of 2023

Strategic highlights: leniolisib progress (2/2)

EEA

JAN 6

Pharming receives positive EMA decision on pediatric investigation plan (PIP) for leniolisib in Europe

AUG 1

Announced EMA Accelerated Assessment granted for adults and adolescents aged 12 and older

OCT 2022

Submission of Marketing Authorisation Application (MAA) to EMA with anticipated validation at the end of October 2022

UK

APR 26

MHRA granted Promising Innovative Medicine (PIM) designation for the treatment of APDS in children 1 year of age to less than 18 years of age

H2 2023

MHRA filing will follow the ECDRP route

Anticipated MHRA decision known in H2 2023

Strategic highlights: OTL-105 & Pompe

Progress continues in preclinical studies

OTL-105

Good progress on developing the lentiviral vector to enhance C1-inhibitor expression, now testing in preclinical HAE disease models

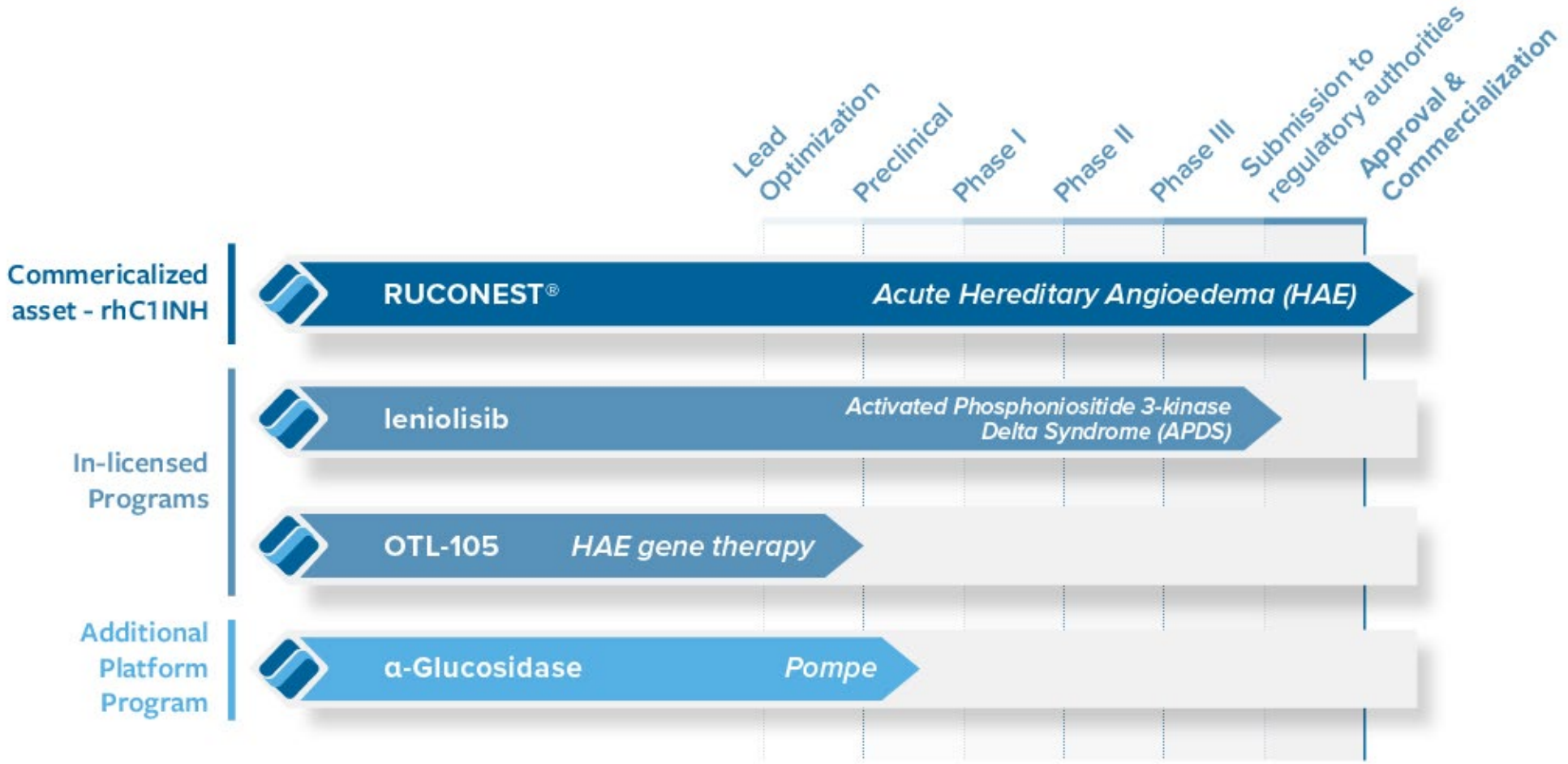
Anticipate providing further updates as we move towards preparing an Investigational New Drug (IND) filing

POMPE

Study into the development of a next-generation alpha-glucosidase therapy for the treatment of Pompe disease is ongoing

Currently engaged in preclinical studies. As and when results from these preclinical studies become available, we will update the market

Pipeline at a glance



Operational highlights: RUCONEST® (conestat alfa)

RUCONEST® sales of US$151.0 million for the first 9 months of 2022

Stable revenues. Will allocate resources to leniolisib with view of accelerating future growth

Safe and effective acute treatment option for hereditary angioedema (HAE)

Single digit growth of revenues expected for the remainder of 2022

Pharming®

CMO

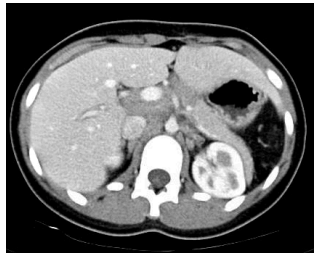
Anurag Relan, MD
Chief Medical Officer

APDS and leniolisib

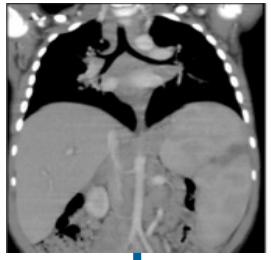
Clinical Features of APDS

Overt Non-malignant Lymphoproliferation

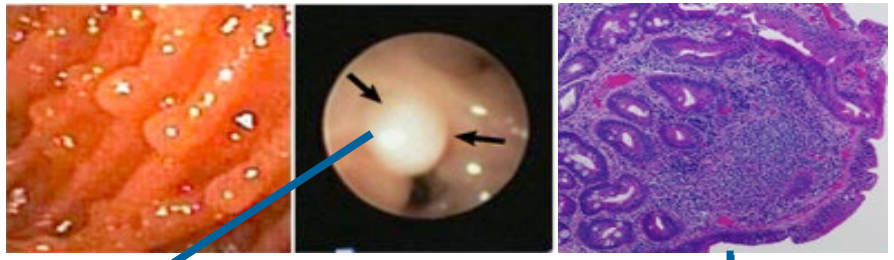
Lymphadenopathy – peripheral, mediastinal, mesenteric



Hepatosplenomegaly



Nodular lymphoid hyperplasia of the airway and GI mucosa



Malignant Lymphoproliferation

Lymphoma – diffuse large B cell, Hodgkin's, marginal zone B cell, MALToma, EBV+

Autoimmunity

Hemolytic anemia, ITP, neutropenia, Evans syndrome

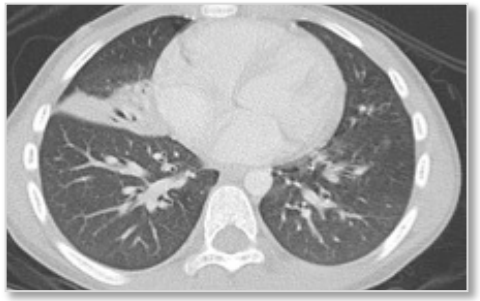
Other autoimmunity

[1-6]

Infections

Encapsulated bacteria
CMV and EBV viremia
CMV lymphadenitis
Varicella zoster
Human papillomavirus
Adenovirus
Molluscum contagiosum

Lung disease



Pneumonia, consolidation, bronchiectasis, interstitial lung disease

GI Disease

Enteropathy
Colitis, IBD-like
Protein-losing enteropathy (PLE)
Eosinophilic esophagitis (EoE)
Intussusception

Liver Disease

Nodular regenerative hyperplasia
Portal hypertension
Sclerosing cholangitis

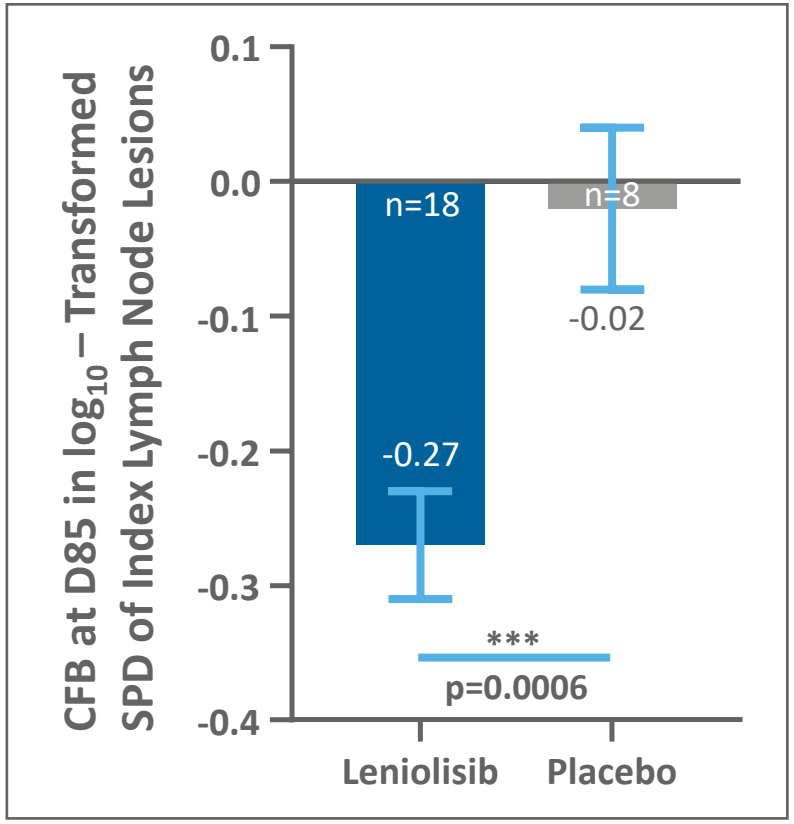
Images courtesy of Dr Gulbu Uzel and the National Institutes of Health. AIHA, autoimmune hemolytic anemia; APDS, activated PI3Kδ syndrome; CMV, cytomegalovirus; EBV, Epstein-Barr virus; GI, gastrointestinal; IBD, inflammatory bowel disease; ITP, immune thrombocytopenic purpura; PASLI, p110δ-activating mutation causing senescent T cells, lymphadenopathy, and immunodeficiency.

1. Lucas CL, et al. *Nat Immunol*. 2014;15(1):88-97. 2. Coulter TI, et al. *J Allergy Clin Immunol*. 2017;139(2):597-606. 3. Elkaim E, et al. *J Allergy Clin Immunol*. 2016;138(1):210-218. 4. Maccari ME, et al. *Front Immunol*. 2018;9:543. 5. Condliffe AM, Chandra A. *Front Immunol*. 2018;9:338. 6. Data on file. Pharming Healthcare Inc. 2022.

Leniolisib reduced lymphadenopathy

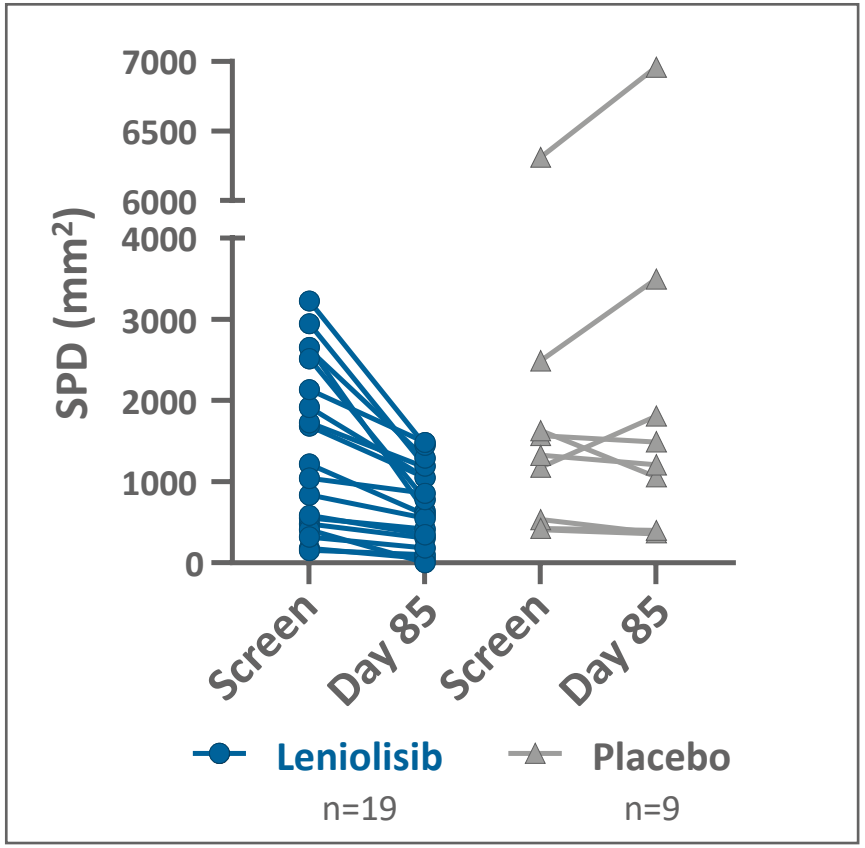
Primary Outcome Analysis*

Change from baseline in index lesions



Individual Index Lesion Sizes

Safety analysis set



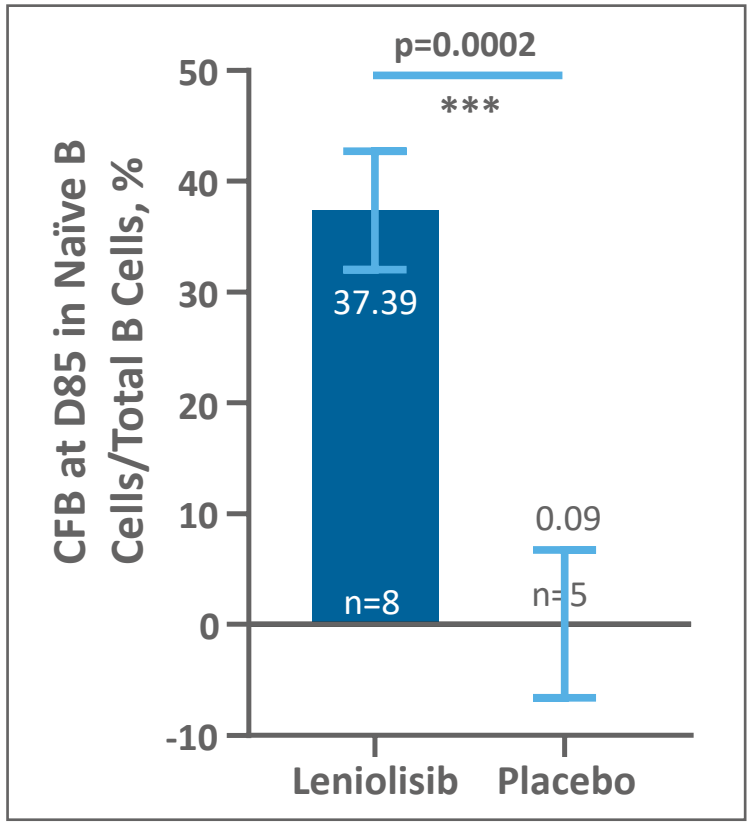
*Data were analyzed using ANCOVA model with treatment as a fixed effect and log_{10}-transformed baseline as a covariate. Use of glucocorticoids and IRT at baseline were both included as categorical (Yes/No) covariates. P-value is 2-sided. Least square means are graphed. Error bars are standard error of the mean. 4 patients from the 31 in the safety analysis were excluded from the PD analysis. An additional patient was excluded from the index lesion analysis because the baseline lung index had fully resolved (0 mm) by D85.

CFB, change from baseline; D, day; PD, pharmacodynamics; SPD, sum of product of diameters; 3D, 3-dimensional. Data on file. Pharming Healthcare Inc. 2022.

Leniolisib Increased the Percentage of Naïve B Cells Out of Total B Cells

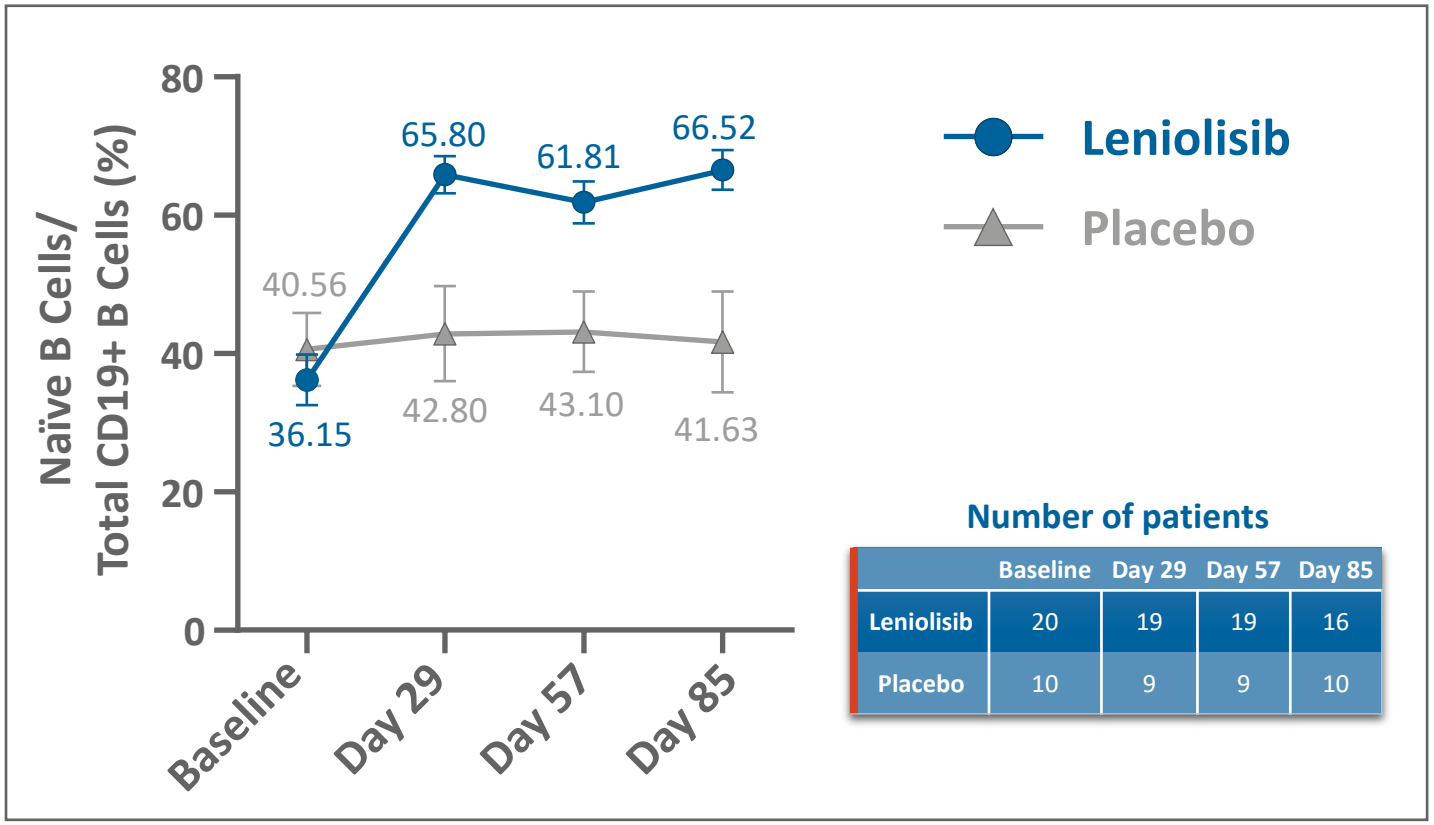
Primary Outcome Analysis*

Change from baseline in naïve B cells



Mean Percentage of Naïve B Cells Over Time

Safety analysis set



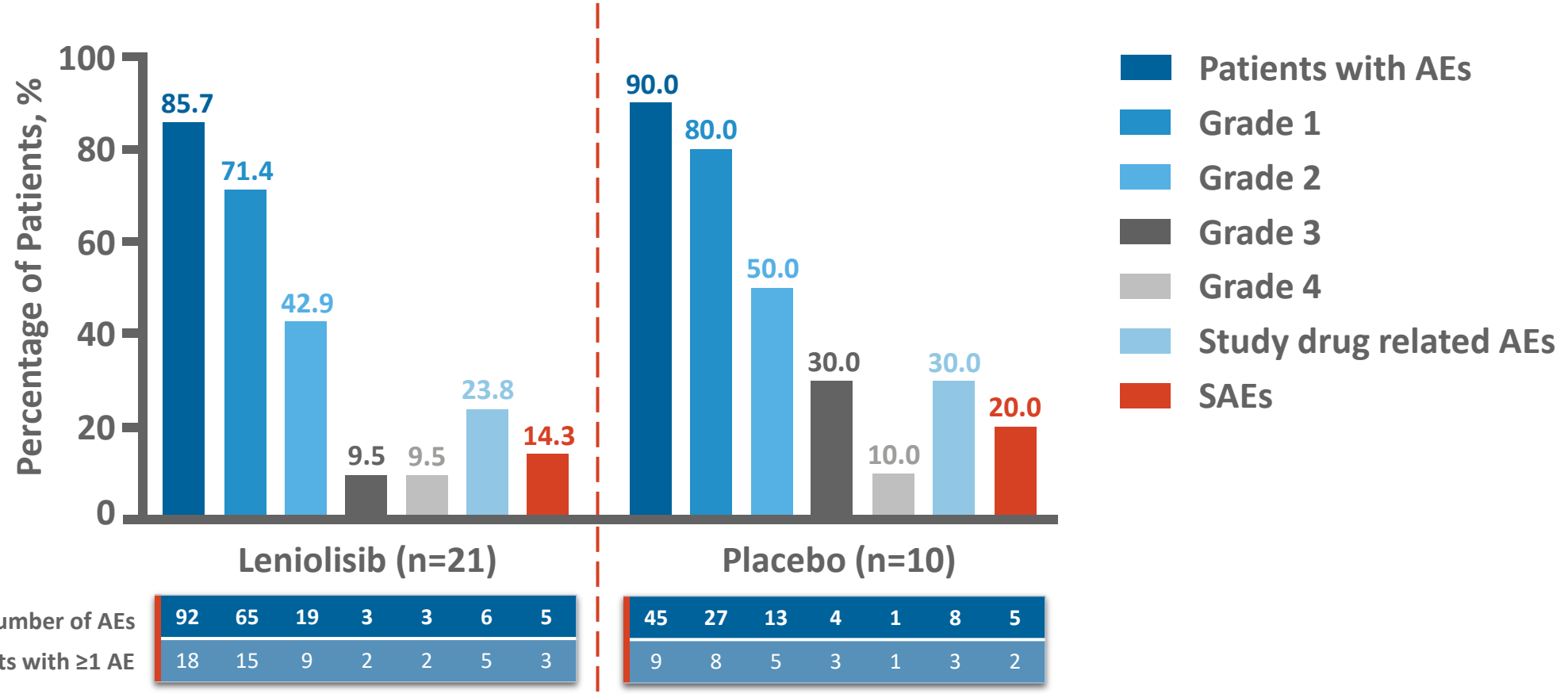
Number of patients

	Baseline	Day 29	Day 57	Day 85
Leniolisib	20	19	19	16
Placebo	10	9	9	10

*Data were analyzed using an ANCOVA model with treatment as a fixed effect and baseline as a covariate. Use of glucocorticoids and IRT at baseline were both included as categorical (Yes/No) covariates. *Baseline* is defined as the arithmetic mean of the baseline and Day 1 values when both are available, and if either baseline or the Day 1 value is missing, the existing value is used. P-value is 2-sided. Least square means are graphed. Error bars are standard error of the mean. Out of 27 patients in the PD analysis set, 13 patients met the analysis requirements, including having a percentage of <48% of naïve B cells at baseline, to form the B-PD analysis set.

Data on file. Pharming Healthcare Inc. 2022.

Leniolisib over three months was well tolerated

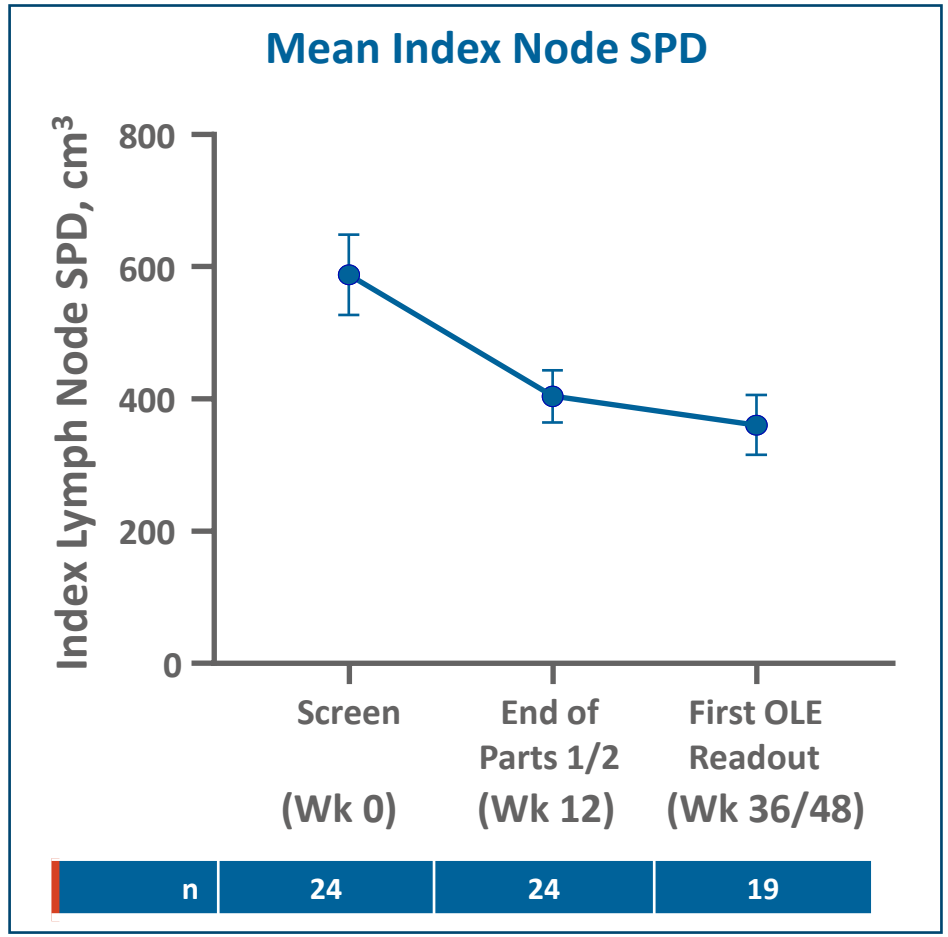


	Leniolisib (n=21)							Placebo (n=10)						
Number of AEs	92	65	19	3	3	6	5	45	27	13	4	1	8	5
Number of patients with ≥1 AE	18	15	9	2	2	5	3	9	8	5	3	1	3	2

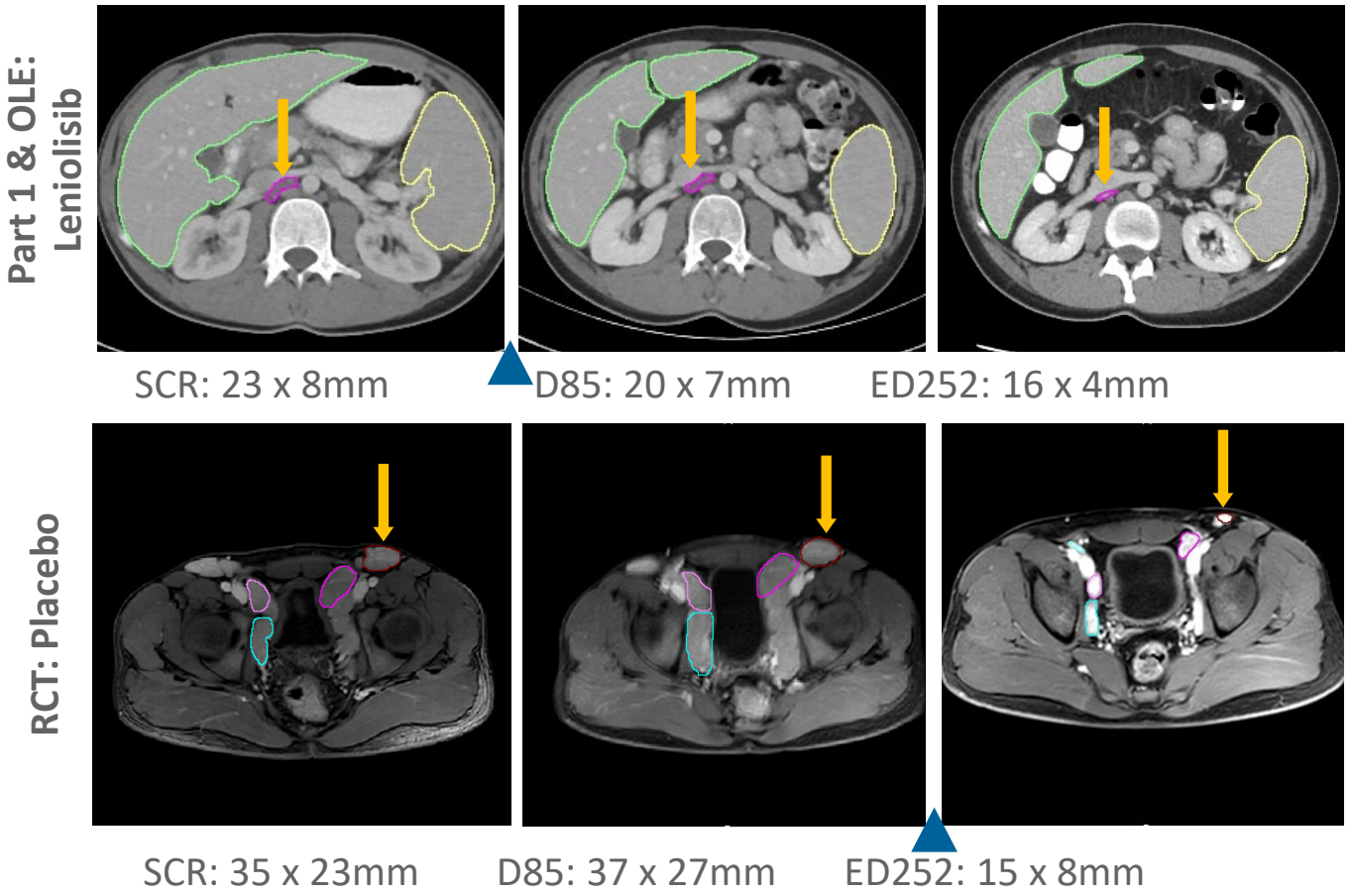
- No deaths (grade 5 AEs) were reported
- No AEs led to discontinuation of study treatment
- No SAEs were related to study treatment, and the incidence of SAEs was lower in the leniolisib group than the placebo group

CTC were used to determine AE grade. If CTC-AE grading did not exist for an AE, the following definitions were used: 1, mild; 2, moderate; 3, severe; 4, life-threatening; 5, death.
AEs, adverse events; CTC, Common Toxicity Criteria; SAEs, serious adverse events. Data on file. Pharming Healthcare Inc. 2022.

Leniolisib Reduced Lymphadenopathy

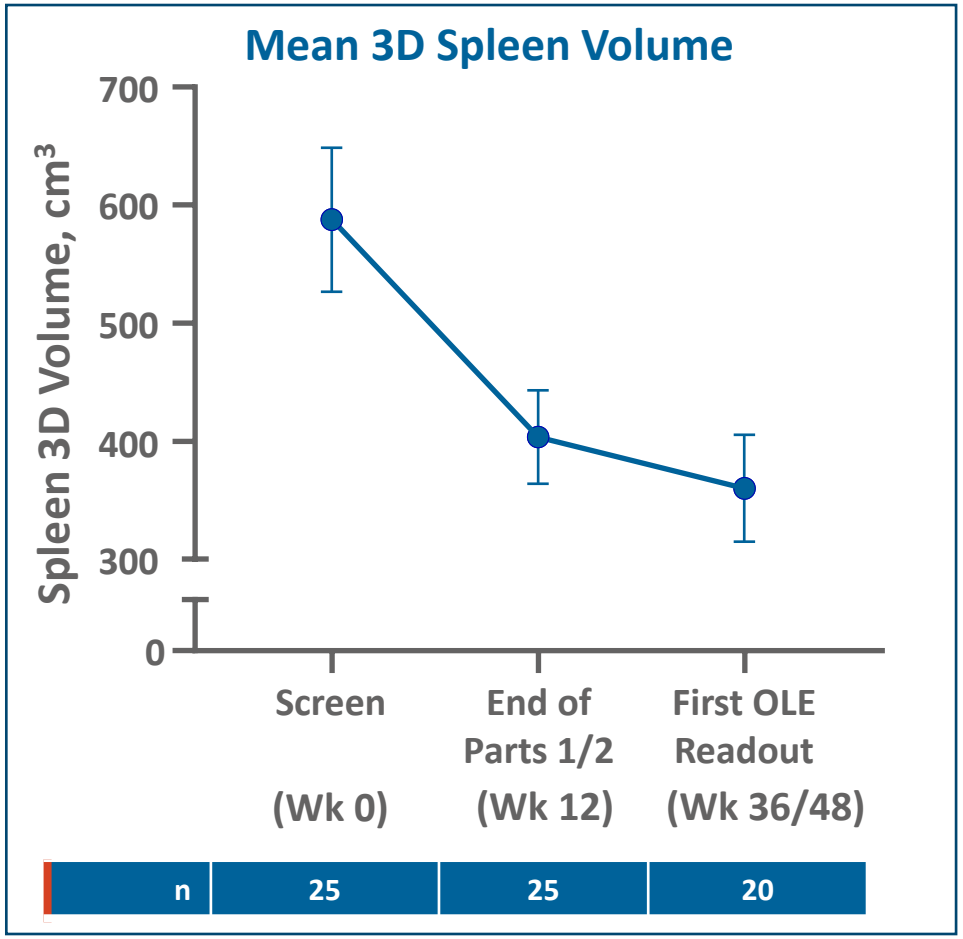


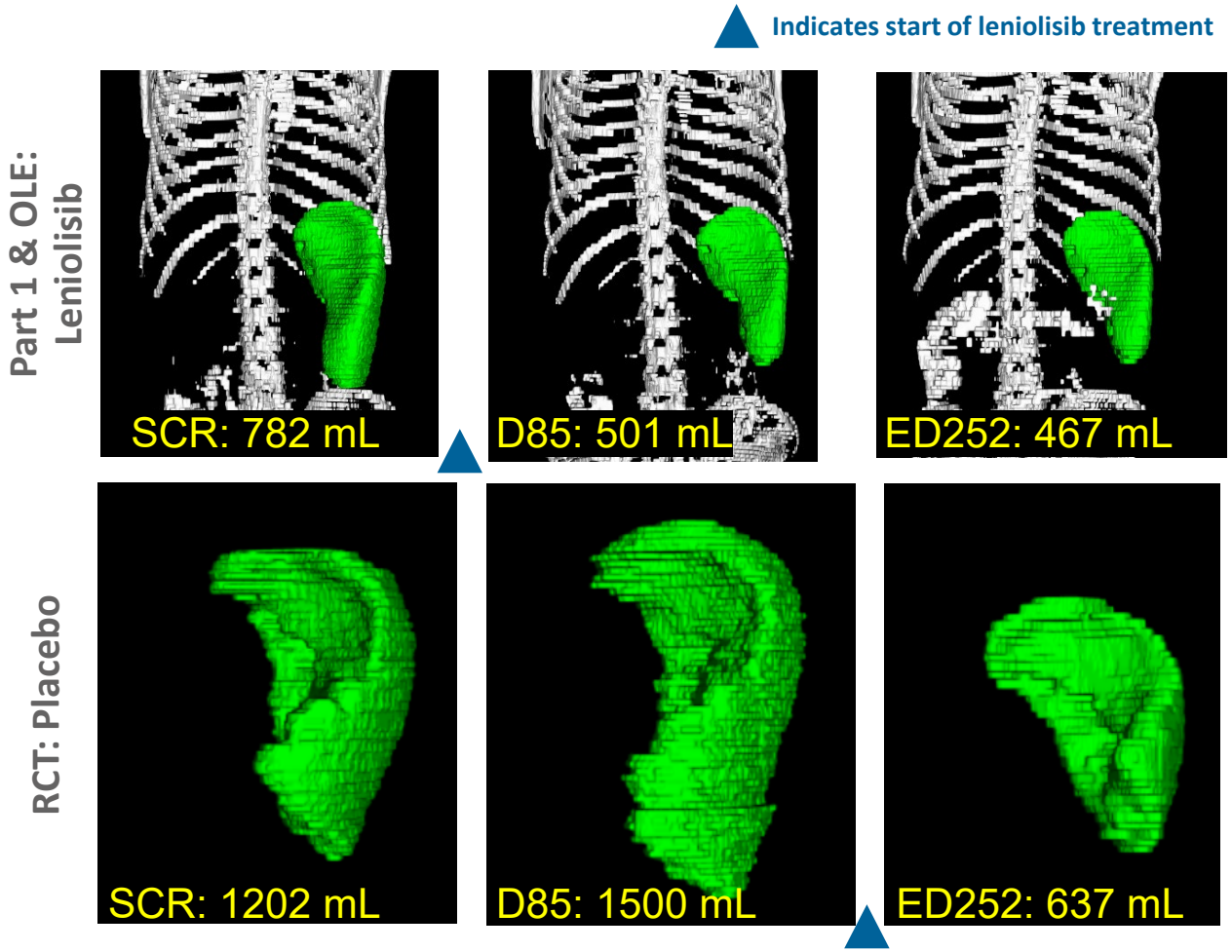
▲ Indicates start of leniolisib treatment



Error bars are standard error of the mean. All patients from parts 1 and 2 of the phase II/III trials with leniolisib exposure and with measurements are included. End of parts 1 and 2 occurred at days 84 and 85, respectively. First OLE readout occurred after an additional 168 or 252. days. D, day; OLE, open-label extension; RCT, randomized controlled trial; SCR, screen; SPD, sum of product diameters; Wk, week.
Data on file. Pharming Healthcare Inc. 2022.

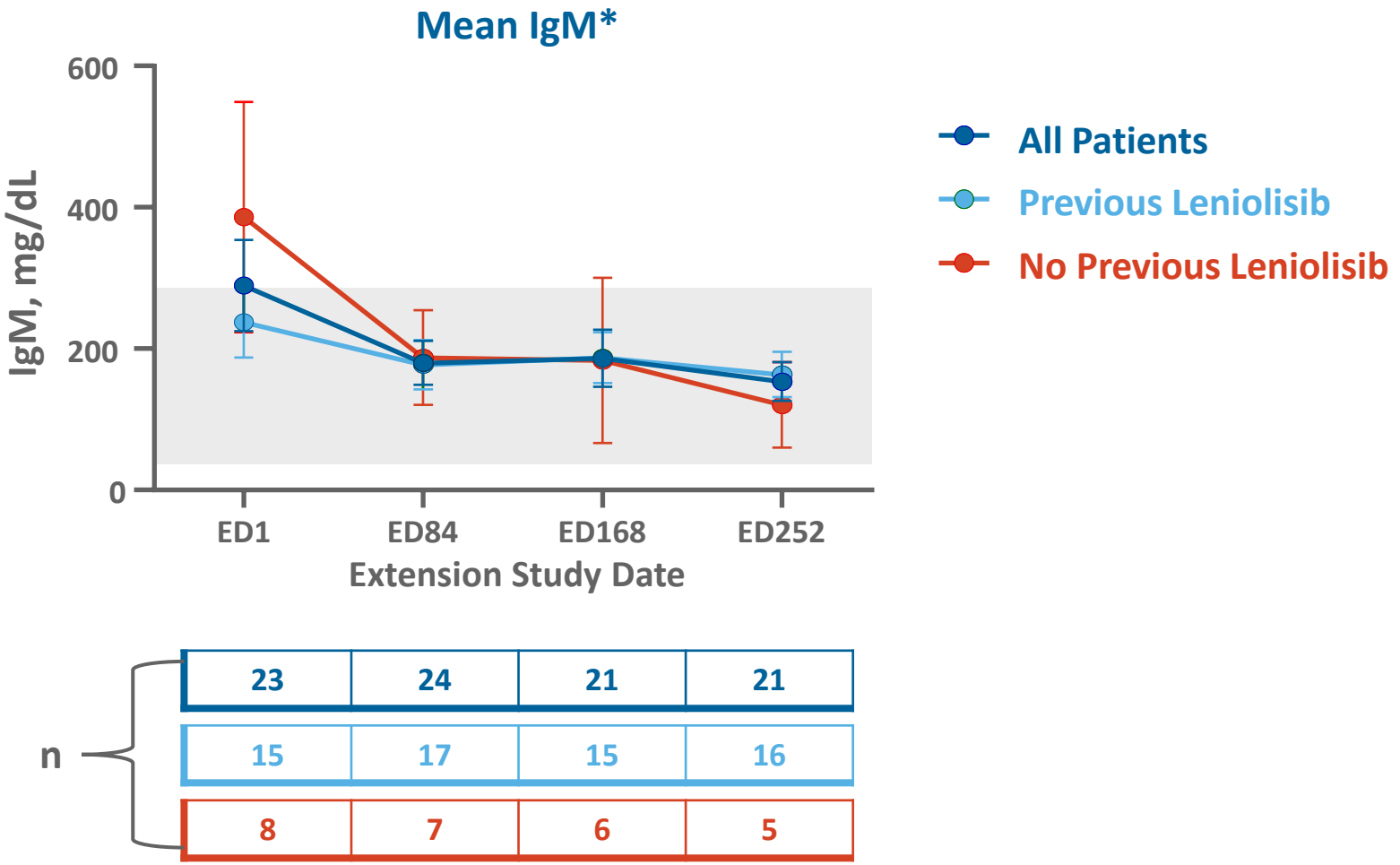
Leniolisib Reduced Spleen Size





Error bars are standard error of the mean. All patients from parts 1 and 2 of the phase II/III trials with leniolisib exposure and with measurements are included. End of parts 1 and 2 occurred at days 84 and 85, respectively. First OLE readout occurred after an additional 168 or 252 days.
Data on file. Pharming Healthcare Inc. 2022.

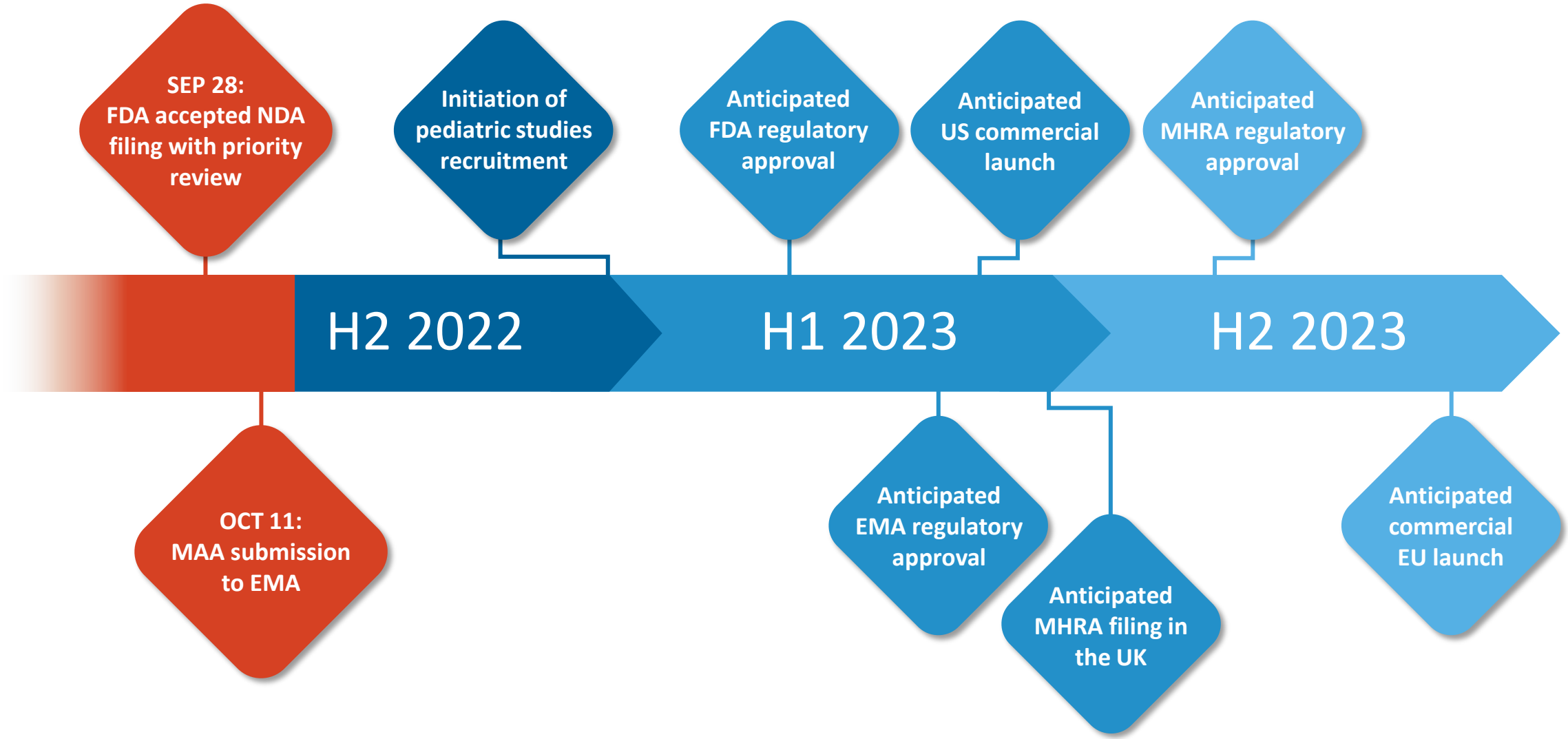
Leniolisib Decreased Elevated IgM



*Excluded 1 patient due to extremely low B-cell count.
Previous Leniolisib includes patients who received leniolisib during the dose-finding trial and RCT. No Previous Leniolisib includes patients who received placebo during the RCT and patients who were enrolled in other PI3Kδ inhibitor trials.
Error bars are standard error of the mean. The gray box indicates the normal range.

Data on file. Pharming Healthcare Inc. 2022.

Upcoming milestones for leniolisib*



*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.

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

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CFO

Jeroen Wakkerman
Chief Financial Officer

Financial highlights

Financial highlights

Total revenues up 3% compared to the first 9M 2021

Gross profit increased by 7% to US$139.7 million, driven by growth in revenues, production efficiencies, and favorable tailwind from currency translation effects

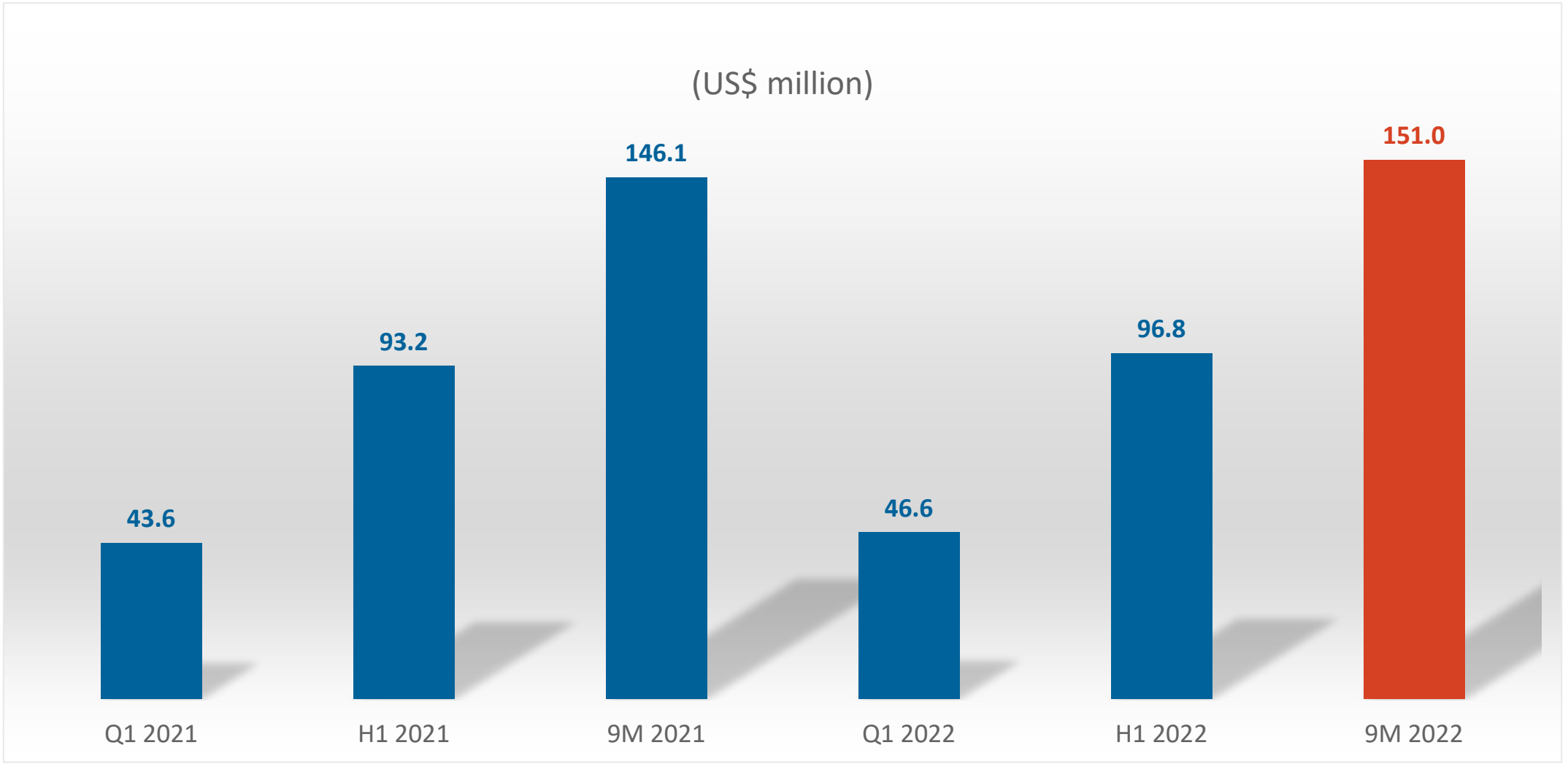
Net profit increased by 104% compared to first 9M 2021, driven by an increase in Other income

Cash and cash equivalents, together with restricted cash, decreased from US$193.0 million at the end of 2021, to US$189.9 million at the end of the third quarter 2022.

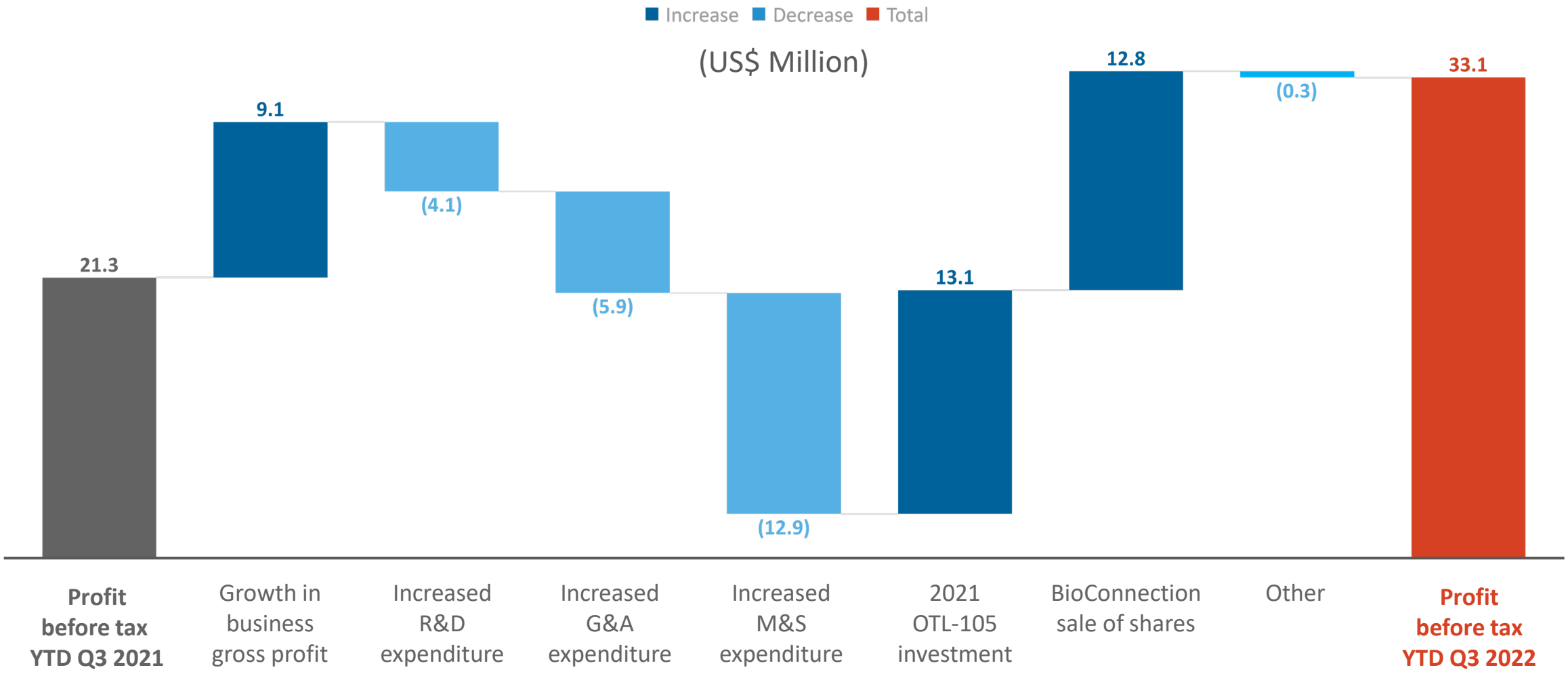
Financial highlights: 9M 2022 vs 9M 2021

TOTAL REVENUES 9M 2021	US$146.1 million	TOTAL REVENUES 9M 2022	US$151.0 million
GROSS PROFIT 9M 2021	US$130.6 million	GROSS PROFIT 9M 2022	US$139.7 million
OPERATING PROFIT 9M 2021	US$15.3 million	OPERATING PROFIT 9M 2022	US$28.4 million
NET PROFIT 9M 2021	US$13.9 million	NET PROFIT 9M 2022	US$28.3 million

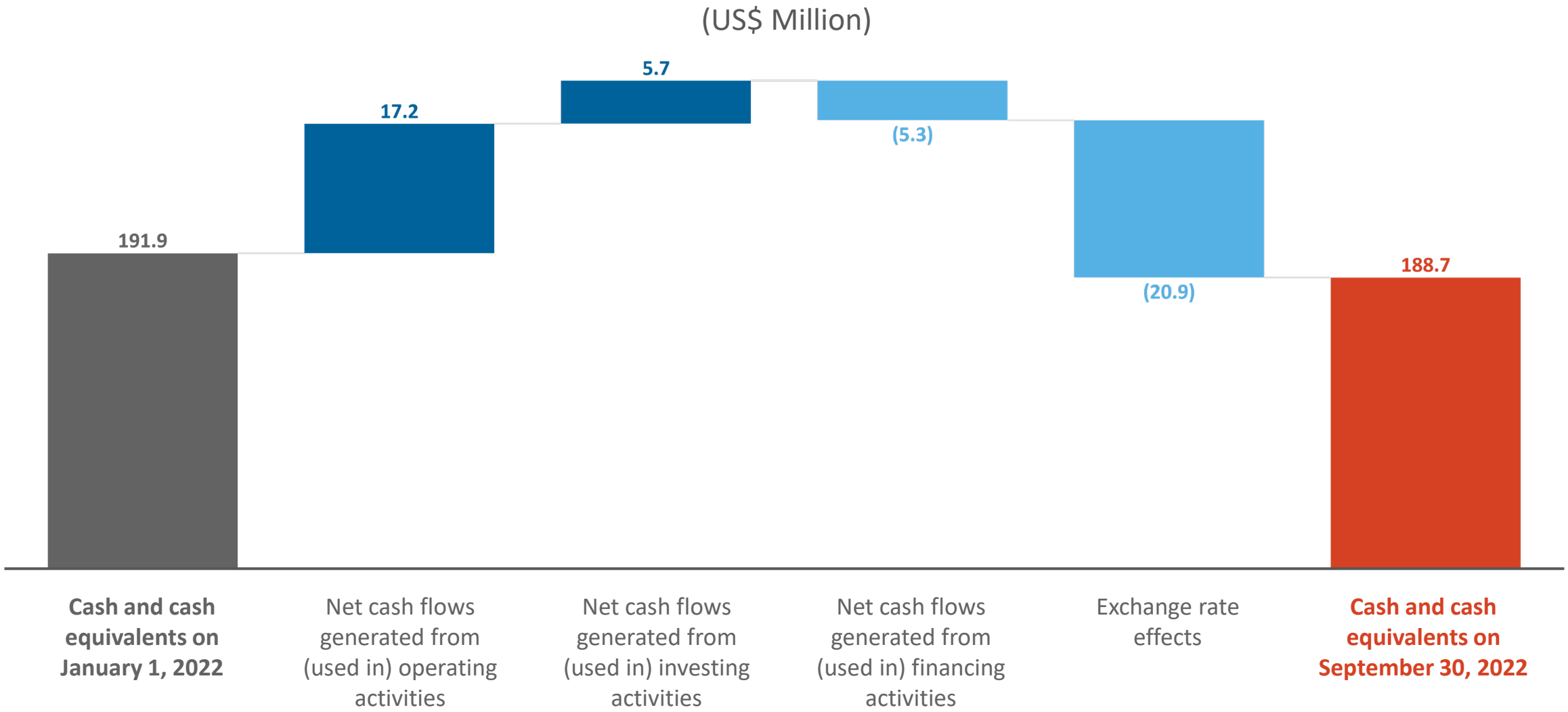
Financial highlights: Revenues 2022 vs 2021



First 9M 2022: Profit before tax Sep 30, 2021 – Sep 30, 2022



First 9M 2022: Cashflow January 1, 2022 – September 30, 2022



Outlook

- **Single digit growth Group revenues from RUCONEST® sales in 2022**
- **Commercial approval of leniolisib from FDA in Q1 2023, with an anticipated launch and commercialization in US in H1 2023.** *subject to positive outcomes of the FDA review
- **Positive opinion of leniolisib from the CHMP, followed by issuance of MAA by European Commission end of H1 2023. Commercial launch in EU markets in H2 2023.**
- **Submit an ECDRP filing for leniolisib to MHRA, after anticipated positive CHMP opinion, MHRA decision expected in H2 2023.**
- **Continue to allocate resources towards the anticipated launch and commercialization of leniolisib with the view of accelerating future growth**
- **Investment and continued focus on potential acquisitions and in-licensing of new, late- stage development opportunities and assets in rare diseases.**

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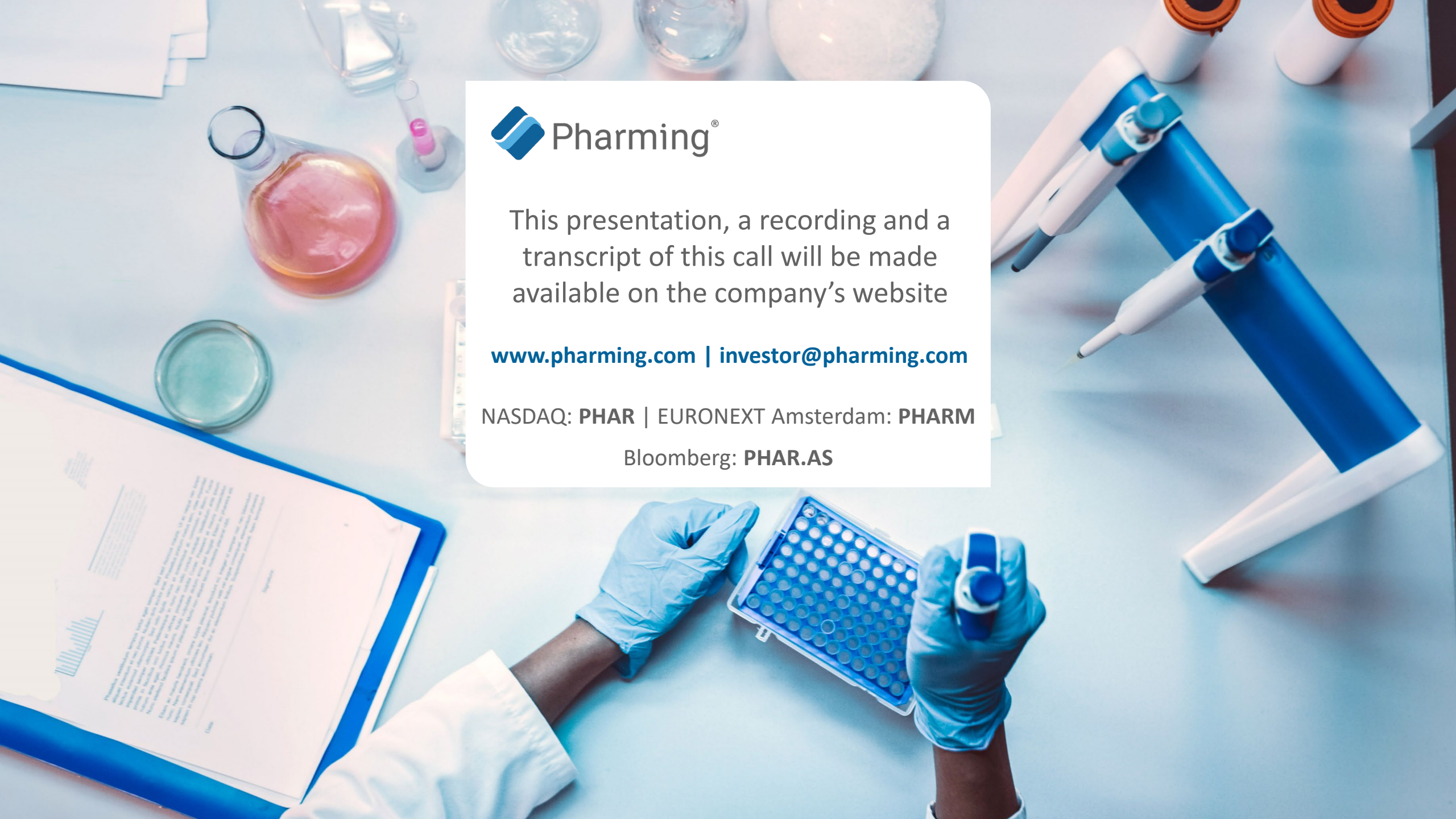
Q&A

Sijmen de Vries, MD
Chief Executive Officer

Anurag Relan, MD
Chief Medical Officer

Jeroen Wakkerman
Chief Financial Officer



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

www.pharming.com | investor@pharming.com

NASDAQ: **PHAR** | EURONEXT Amsterdam: **PHARM**

Bloomberg: **PHAR.AS**

Statement of profit and loss

Amounts in US$ '000	YTD 2022	YTD 2021
Revenues	**151.001**	**146.101**
Costs of sales	(11.288)	(15.500)
Gross profit	**139.712**	**130.601**
Other income	15.602	1.808
Research and development	(41.639)	(37.580)
OTL-105 in-licensing	0	(13.105)
General and administrative	(28.446)	(22.510)
Marketing and sales	(56.819)	(43.880)
Other Operating Costs	**(126.904)**	**(117.075)**
Operating profit	**28.410**	**15.334**
Fair value gain (loss) on revaluation derivatives	0	59
Other finance income	9.297	9.907
Other finance expenses	(3.978)	(4.466)
Finance cost net	**5.319**	**5.500**
Share of net profits in associates using the equity method	**(660)**	**511**
Profit before tax	**33.069**	**21.345**
Income tax credit (expense)	(4.765)	(7.412)
Profit for the year	**28.304**	**13.933**
Basic earnings per share (US$)	0.043	0.022
Fully-diluted earnings per share (US$)	0.040	0.018

Balance sheet – assets

Amounts in US$ '000	30 September 2022	31 December 2021
Non-current assets		
Intangible assets	70.123	83.834
Property, plant and equipment	10.812	13.222
Right-of-use assets	16.970	19.943
Long-term prepayments	210	194
Deferred tax assets	21.187	21.216
Investments accounted for using the equity method	2.845	7.201
Investment in equity instruments designated as at FVTOCI	545	1.449
Investment in debt instruments designated as at FVTPL	7.386	0
Restricted cash	197	812
Total non-current assets	**130.275**	**147.871**
Current assets		
Inventories	33.506	27.310
Trade and other receivables	28.828	29.983
Restricted cash	1.011	227
Cash and cash equivalents	188.703	191.924
Total current assets	**252.048**	**249.444**
Total assets	**382.323**	**397.315**

Balance sheet – liabilities

Equity		
Share capital	7.482	7.429
Share premium	459.450	455.254
Legal reserves	(24.145)	3.400
Accumulated deficit	(242.533)	(273.167)
Shareholders' equity	**200.254**	**192.916**
Non-current liabilities		
Convertible bonds	120.005	139.007
Lease liabilities	15.227	18.456
Other financial liabilities	143	165
Total non-current liabilities	**135.375**	**157.628**
Current liabilities		
Convertible bonds	1.627	1.879
Derivative financial liabilities	0	0
Trade and other payables	42.744	42.473
Lease liabilities	2.323	2.419
Total current liabilities	**46.694**	**46.771**
Total equity and liabilities	**382.323**	**397.315**

Cash flow (1/2)

Amounts in $'000	YTD 2022	YTD 2021
Profit before tax	**33.069**	**21.345**
Non-cash adjustments:		
Depreciation, amortization, impairment	6.216	6.867
Equity settled share based payments	4.522	5.706
Fair value gain (loss) on revaluation of derivatives	0	(59)
Gain on disposal of investment in associate	(12.382)	0
Other finance income	(9.296)	(9.907)
Other finance expense	3.978	4.466
Share of net profits in associates using the equity method	660	(511)
Other	0	272
Operating cash flows before changes in working capital	**26.767**	**28.179**
Changes in working capital:		
Inventories	(6.196)	(3.941)
Trade and other receivables	1.155	3.092
Payables and other current liabilities	272	(5.514)
Restricted Cash	169	42
Total changes in working capital	**(4.600)**	**(6.321)**
Interest received	31	51
Income taxes paid	(4.975)	0
Net cash flows generated from (used in) operating activities	**17.223**	**21.909**

Cash flow (2/2)

Capital expenditure for property, plant and equipment	(1.071)	(7.451)
Investment intangible assets	(591)	(1.544)
Investment in equity instruments designated as at FVTOCI	0	(4.589)
Investment in associate	7.384	0
Acquisition of license	0	(1.593)
Net cash flows used in investing activities	**5.722**	**(15.177)**
Repayment on loans and borrowings	0	0
Payment on contingent consideration	0	(25.000)
Payment of lease liabilities	(2.385)	(2.476)
Proceeds of issued convertible bonds	0	0
Interests on loans and leases	(3.999)	(4.493)
Proceeds of equity and warrants	1.124	4.237
Net cash flows generated from (used in) financing activities	**(5.260)**	**(27.732)**
Increase (decrease) of cash	**17.685**	**(21.000)**
Exchange rate effects	(20.906)	(835)
Cash and cash equivalents at 1 January	191.924	205.159
Total cash and cash equivalents at 30 September	**188.703**	**183.324**