



Pharming Group N.V.
H.C Wainwright 24th Annual
Investment Conference

September 13, 2022

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



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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One commercialized asset: RUCONEST® (conestat alfa) for the treatment of acute hereditary angioedema (HAE)



Founded in 1988 in Leiden, the Netherlands. Pharming Group N.V. has a rich history within the Science Park.



Global headquarters: Leiden, the Netherlands
US headquarters: Warren, New Jersey



Active in over 30 markets, the largest markets include: United States, Europe, United Kingdom, Middle East & North Africa



EURONEXT Amsterdam: PHARM: Since 1999
Nasdaq: PHAR: Since 2020



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

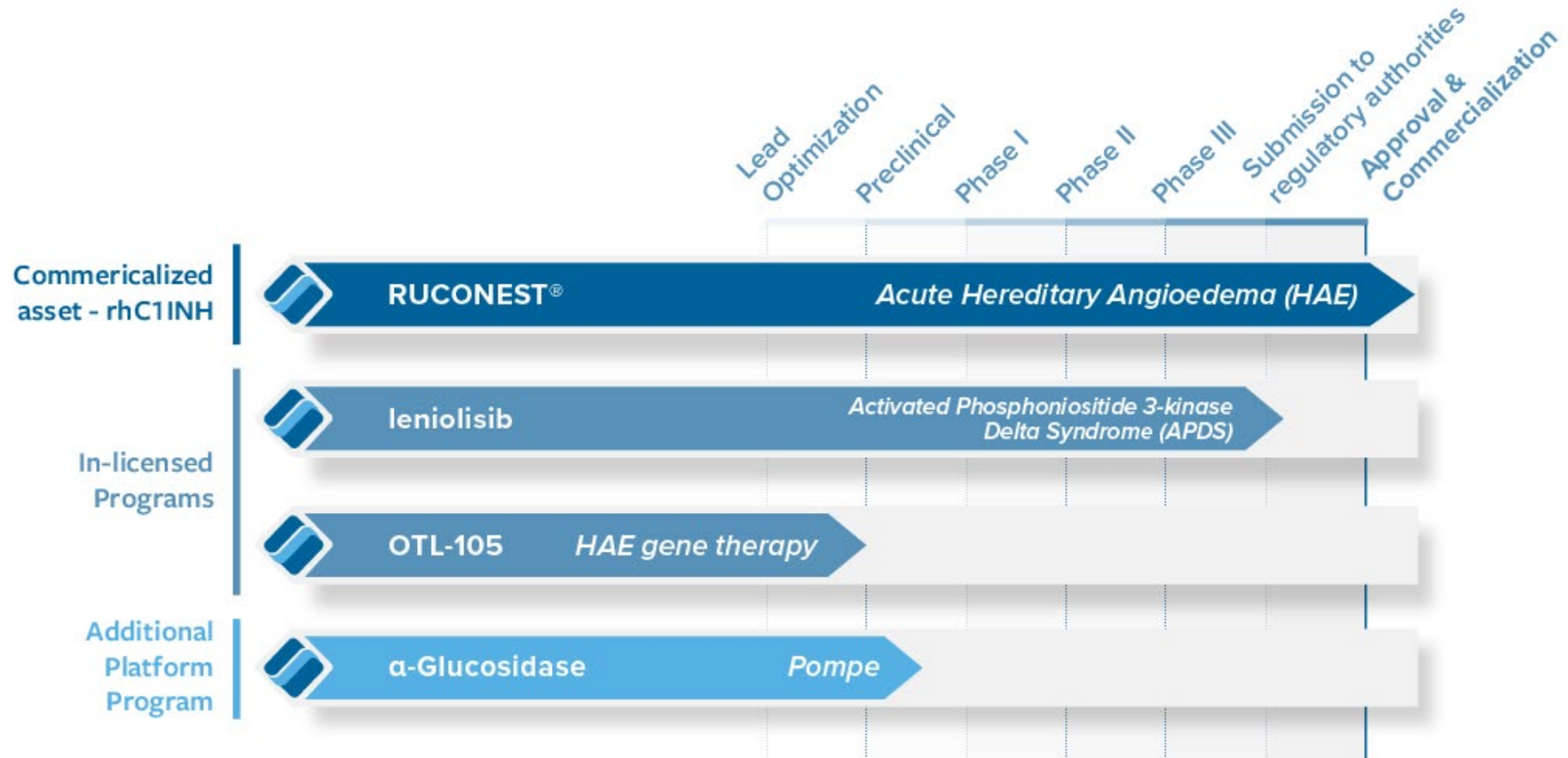


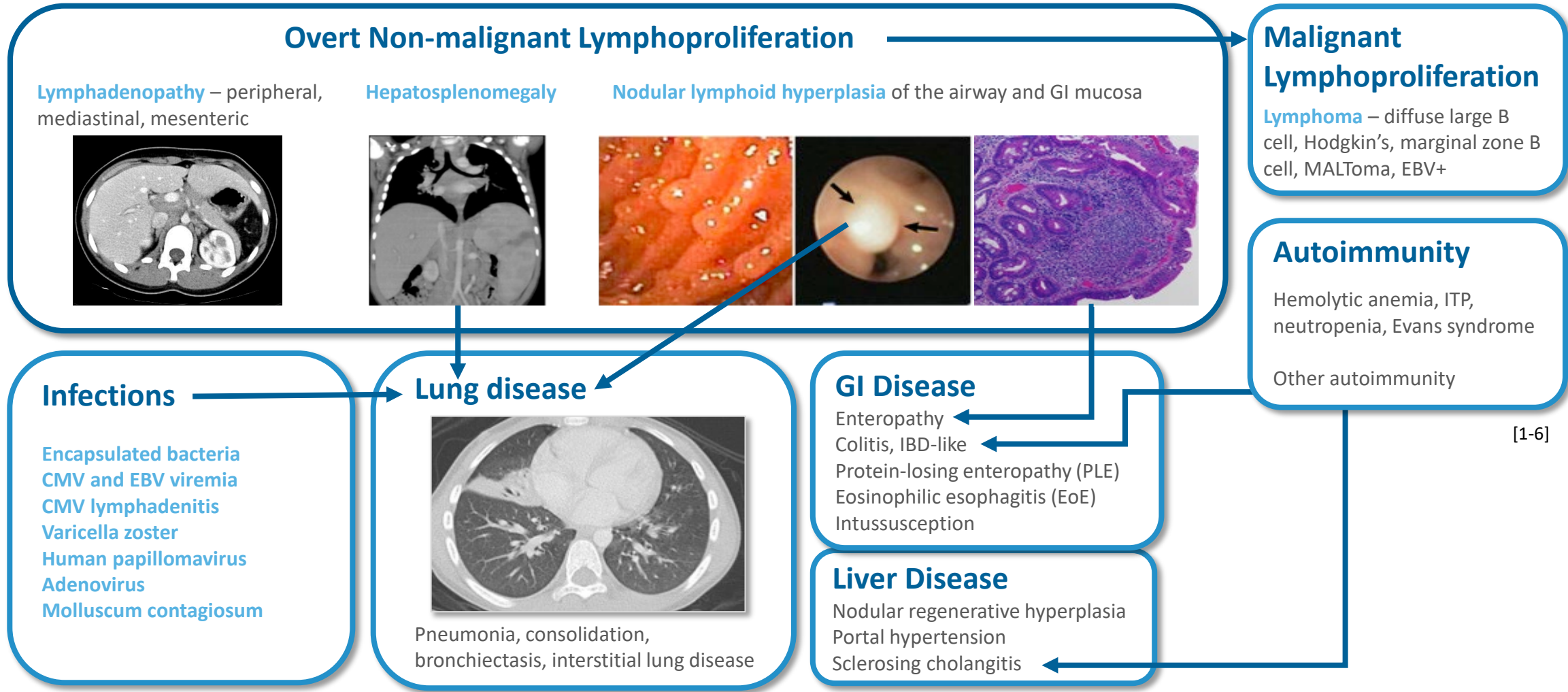
Ongoing pipeline development and management of rare disease assets

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

Pipeline at a glance



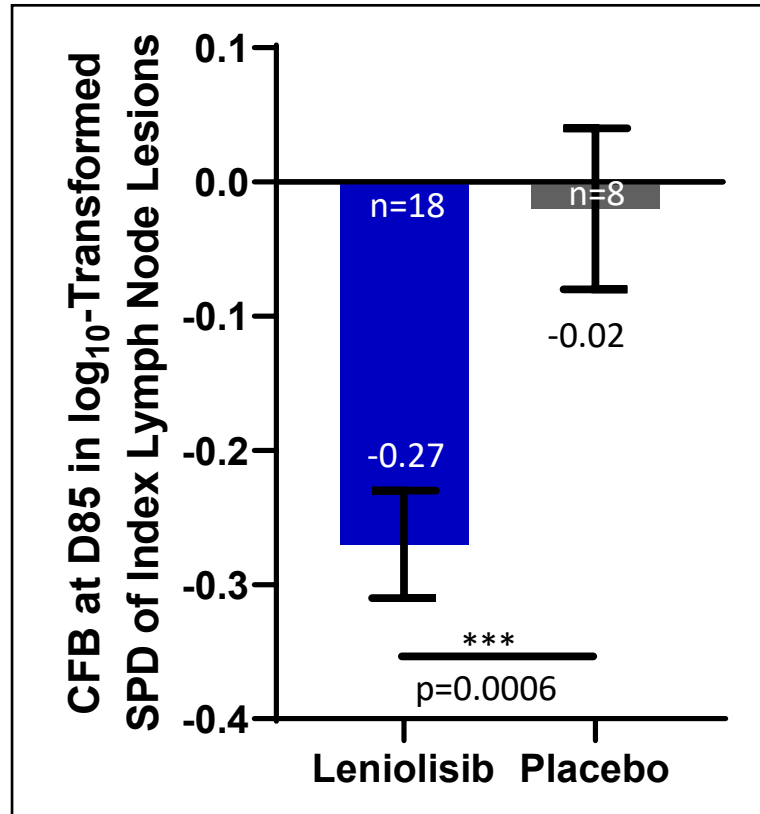


Images courtesy of Dr Gulbu Uzel and the National Institutes of Health. AIHA, autoimmune hemolytic anemia; APDS, activated PI3Kδ syndrome; CMV, cytomegalovirus; EBV, Epstein-Barr virus; GI, gastrointestinal; IBD, inflammatory bowel disease; ITP, immune thrombocytopenic purpura; PASLI, p110δ-activating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

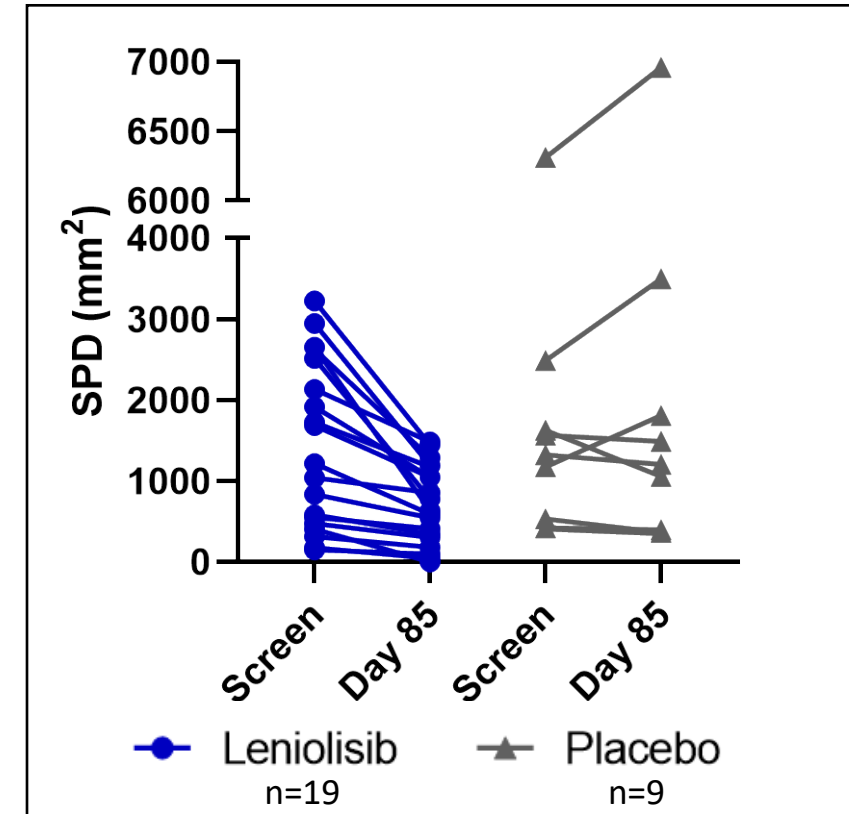
Primary Outcome Analysis*

Change from baseline in index lesions



Individual Index Lesion Sizes

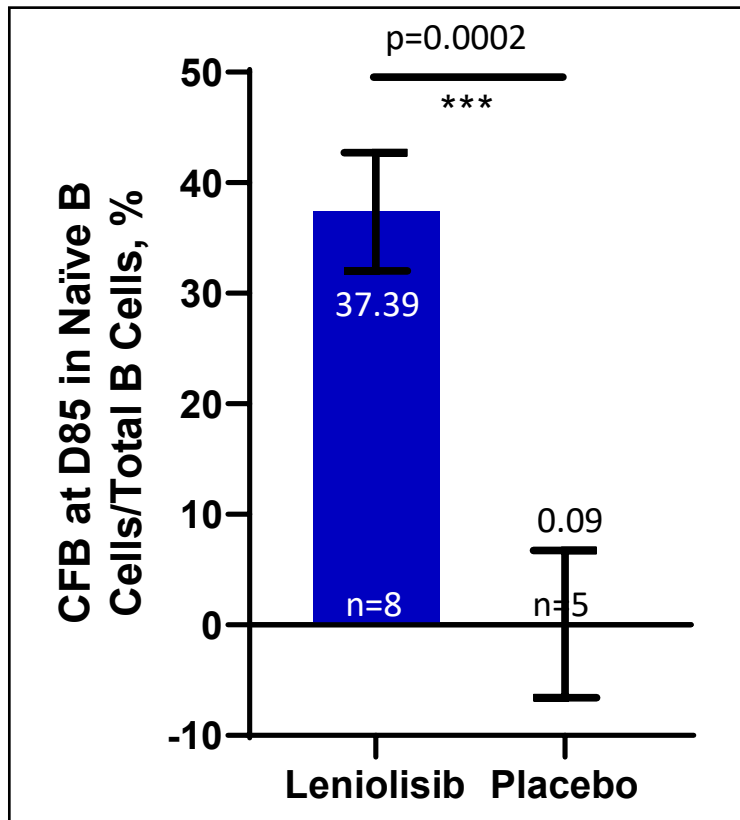
Safety analysis set



*Data were analyzed using ANCOVA model with treatment as a fixed effect and \log_{10} -transformed baseline as a covariate. Use of glucocorticoids and IVIG 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.

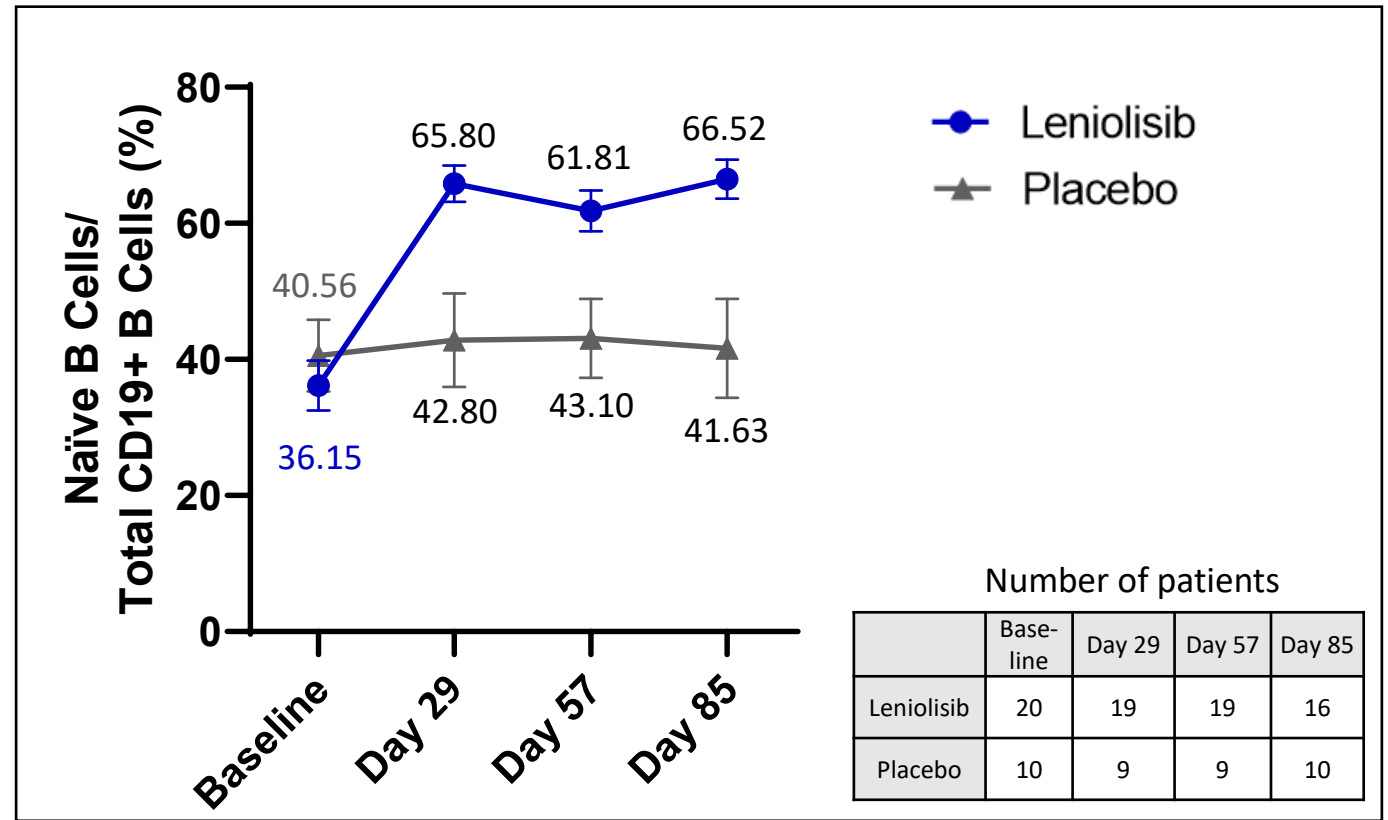
Primary Outcome Analysis*

Change from baseline in naïve B cells



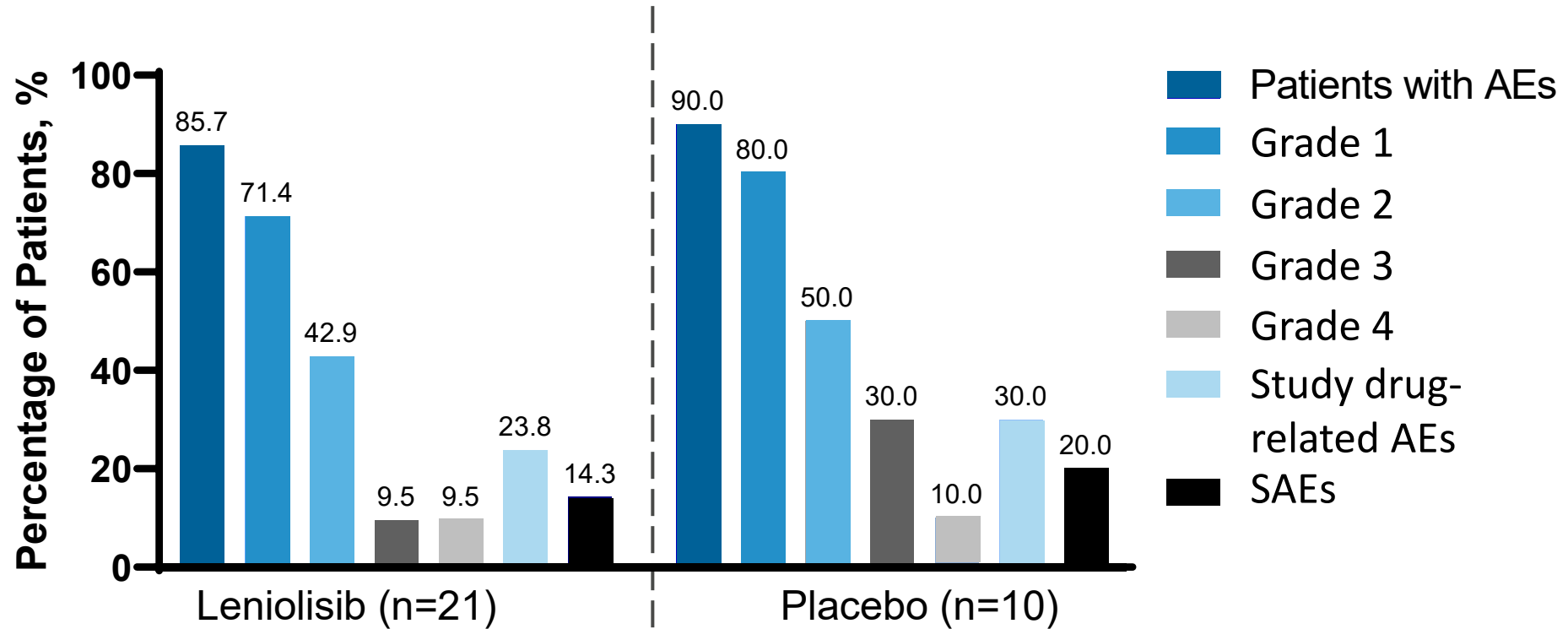
Mean Percentage of Naïve B Cells Over Time

Safety analysis set



*Data were analyzed using an ANCOVA model with treatment as a fixed effect and baseline as a covariate. Use of glucocorticoids and IVIG 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.

Leniolisib over three months was well tolerated



Number of AEs

92	65	19	3	3	6	5
18	15	9	2	2	5	3

Number of patients with ≥1 AE

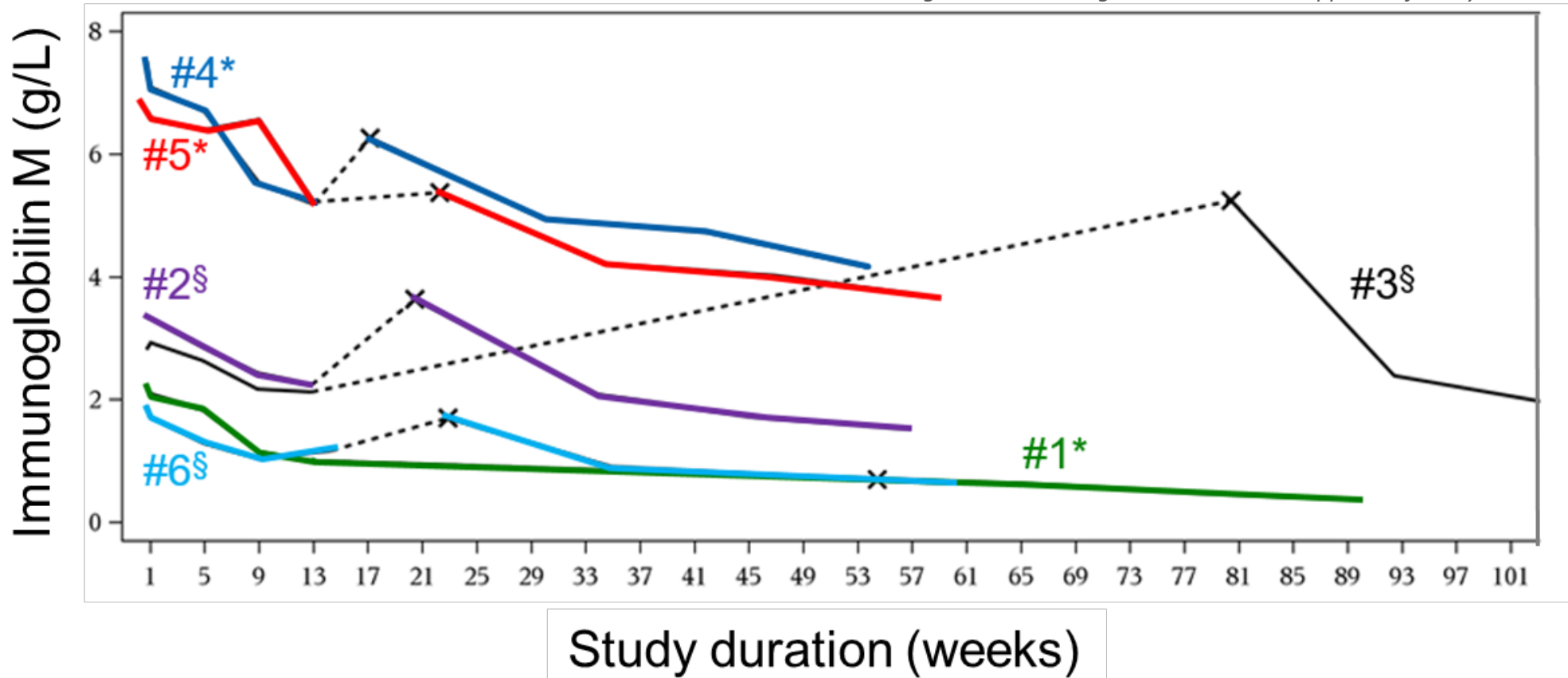
45	27	13	4	1	8	5
9	8	5	3	1	3	2

- ◆ 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

Long term leniolisib results (N=6)

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



Patients have stopped (*) or decreased (\$) immunoglobulin supplementation as a reflection of the normalization of their B cell function. Dashed lines indicate patient not on treatment



We remain on track for the commercial approval of leniolisib in the US, UK and the EU



Filing of New Drug Authorization with the FDA

JUL 29



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

JAN 6



International Classification of Diseases, 10th Revision, Clinical Modification (ICD-10-CM) by the US CDC for APDS, will be effective starting October 1, 2022

AUG 2



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

APR 26



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

AUG 1



We remain on track for the anticipated commercial approval from the FDA in Q1 2023, with an anticipated launch and commercialization soon after

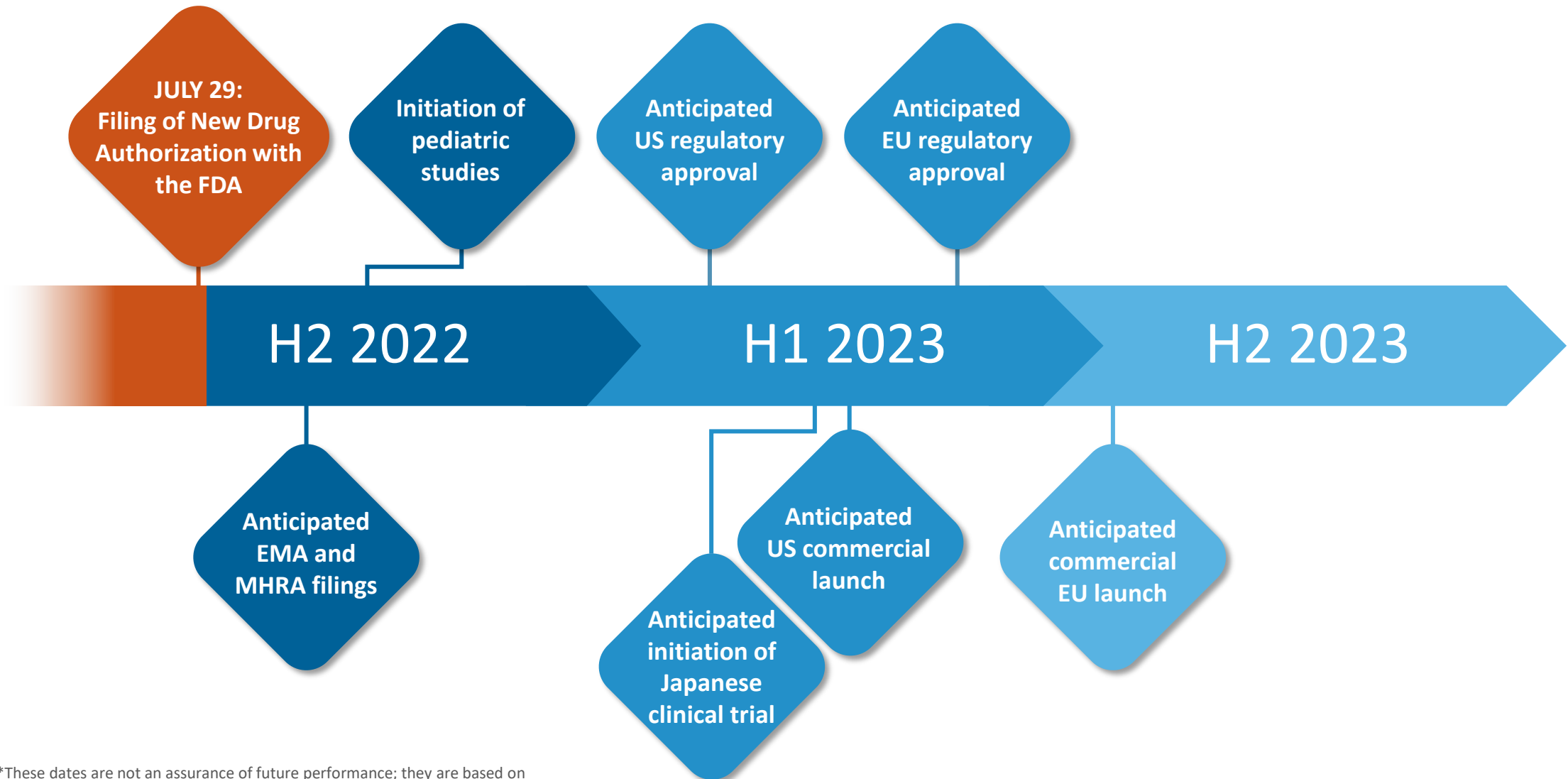
Q1 2023



Remain on track for regulatory filings for both EMA and MHRA in the second half of 2022

H2 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.



RUCONEST® sales of US\$198.9 million in FY 2021



RUCONEST® sales growth supported by an increase in physicians prescribing and number of patients



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



Market representation (total revenues)
US sales – 97% | EU & RoW – 3%



Single digit growth expected to continue for remainder of 2022



Financial Highlights H1 2022





Total revenues up 4% compared to H1 2021



Net profit increased by 33% compared to H1 2021



Positive cash flows, offset by exchange rate effect



Stake held by Pharming in BioConnection reduced, received a one-off US\$7.5 million cash payment and recognized gain of US\$12.8 million.

Financial highlights: H1 2022

**TOTAL REVENUES
H1 2021**

US\$93.2 million



**TOTAL REVENUES
H1 2022**

Increased by 4%
to US\$96.8 million



**GROSS PROFIT
H1 2021**

US\$83.8 million



**GROSS PROFIT
H1 2022**

Increased by 5%
to US\$87.9 million



**OPERATING PROFIT
H1 2021**

US\$17.2 million



**OPERATING PROFIT
H1 2022**

Increased by 20%
to US\$20.6 million



**NET PROFIT
H1 2021**

US\$14.4 million

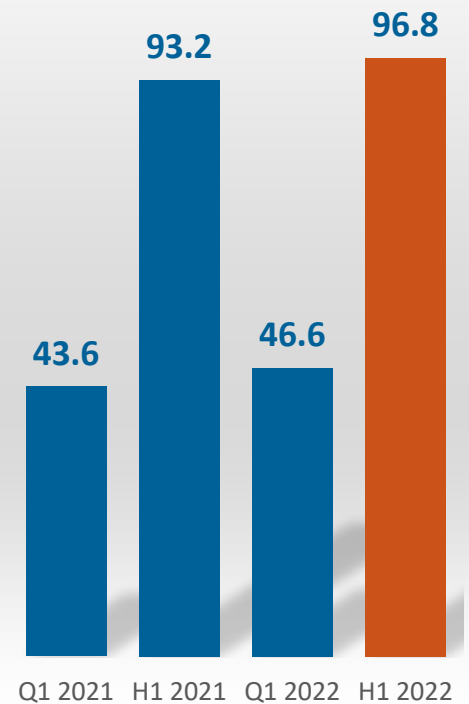


**NET PROFIT
H1 2022**

Increased by 33%
to US\$19.2 million



(US\$ Million)





Single digit growth in Group revenues from RUCONEST[®] sales, quarterly fluctuations are expected.



On track for leniolisib regulatory filings to EMA and UK MHRA in H2 2022.



Commercial approval of leniolisib from FDA in Q1 2023, with an anticipated launch and commercialization in US.

*subject to positive outcomes of the FDA review and granting of a Priority Review



Continue to allocate resources towards the anticipated launch and commercialization of leniolisib.



Investment & continued focus on potential acquisitions and in-licensing of new, late-stage development opportunities and assets in rare diseases.



This presentation will be made available on
the company's website.

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

Bloomberg: **PHAR.AS**