Pharming Group reports financial results for the first quarter of 2023

- **FDA approval and commercial launch of Joenja® (leniolisib) in the U.S.**
- **Joenja® launch off to strong start with 23 U.S. patients on paid therapy**
- **Revenues decreased by 9% to US$42.5 million, compared to the first quarter of 2022, primarily due to temporary reimbursement disruptions for some patients on government insurance programs. This issue, affecting all of the HAE market, has since resolved**
- **Continue to anticipate low single digit growth in annual revenues from RUCONEST® in 2023**
- **Continued significant investments made in leniolisib launch preparations and organizational structure**
- **Overall cash balance of US$184.8 million at the end of the quarter**

Leiden, The Netherlands, May 11, 2023: Pharming Group N.V. ("Pharming" or "the Company") (Euronext: PHARM/Nasdaq: PHAR) presents its preliminary, unaudited financial report for the first quarter ended March 31, 2023.

Chief Executive Officer, Sijmen de Vries commented:

"In the first quarter of 2023, we reached an important milestone with the approval and launch of Joenja® (leniolisib), the first and only approved treatment for patients with APDS in the United States. With this approval, U.S.-based patients living with APDS now have access to the medication to normalize their underlying immune function, and Pharming is one step closer to its goal of becoming a leading global rare disease company dedicated to patient communities with unmet medical needs.

The launch of Joenja® is off to a strong start. In April, we announced that the first commercial product had been shipped to patients with payor reimbursement and we can confirm that we now have 23 patients on paid therapy.

Turning to RUCONEST®, as we guided on our 2022 full year results call on March 16, we continue to anticipate low single digit revenue growth with quarterly fluctuations. In the first quarter, there were a number of fluctuations that impacted RUCONEST® revenues and the HAE market across acute and prophylactic products. These factors were mainly due to disruptions in reimbursement for some patients on government insurance programs, which delayed shipments.

These impacts to the business have since resolved and we can confirm that we have seen a recovery and catch up in revenues. Additionally, we are observing strong underlying in-market demand for RUCONEST® and have a high number of new patient enrollments in the U.S. as we entered the second quarter of this year.

Looking ahead, we continue to progress leniolisib through regulatory pathways in Europe, the U.K. and Japan and expect a positive opinion from the European regulator in the second half of 2023, with Europe and U.K. approvals following approximately two months later.

We also began the first of two pediatric clinical trials for children with APDS, whom we believe account for some 25% of U.S.-based APDS patients. The first trial for ages 4 to 11 began in February with the second, for children ages 1 to 6, to follow in the third quarter of this year. These pediatric trials, as well as the development of further leniolisib indications, will add to Pharming's pipeline.

Finally, with a focus on rare disease assets, we are continuously investigating in-licensing and acquisition opportunities which can further enhance our portfolio."

First Quarter and subsequent highlights

Commercialized assets

U.S. FDA Approval of Joenja® (leniolisib) – the first and only treatment approved for APDS

On March 24, 2023, Pharming announced that the US Food and Drug Administration (FDA) approved Joenja® (leniolisib) for the treatment of activated phosphoinositide 3-kinase delta (PI3Kδ) syndrome (APDS) in adult and pediatric patients 12 years of age and older. Joenja®, an oral, selective PI3Kδ inhibitor, is the first and only treatment approved in the U.S. for APDS. The FDA evaluated the New Drug Application (NDA) for Joenja® under priority review and approved the drug based on findings from a multinational, triple-blind, placebo-controlled, randomized Phase II/III clinical trial.

With the approval of Joenja®, as a treatment for primary immunodeficiency, the FDA granted Pharming a priority review voucher ("PRV"). Pursuant to the terms of Pharming's 2019 exclusive license agreement with Novartis for leniolisib, Novartis has the right to purchase the PRV from Pharming for a small minority share of the value of the PRV. Pursuant to the agreement with Novartis, the first commercial sale of Joenja® in the second quarter of 2023 triggered a US$10 million milestone payment by Pharming to Novartis and the regulatory approval for APDS triggered a US$0.5 million milestone payment by Pharming to another party in the first quarter of 2023. Pharming is obligated to make certain additional milestone payments to

Novartis totaling up to US$190 million upon the achievement of leniolisib sales milestones and tiered royalty payments to Novartis calculated as low-teen to high-teen percentages of leniolisib net sales.

Additional information on the PRV, as well as milestone and royalty payments can be found in our 2022 Annual Report or in the Form 20-F filed with the SEC.

Joenja® U.S. Commercial Launch

Based on available literature, Pharming estimates that over 1,500 patients are affected by APDS in the U.S., Europe, U.K., Japan, Canada and Australia. Pharming has already identified over 500 of these patients, of which approximately 200 are U.S.-based. Of that, approximately 75% are U.S.-based patients over the age of 12 and are currently eligible for treatment with Joenja®.

The first commercial shipments of Joenja® to APDS patient in the U.S., with payor reimbursements, were delivered approximately two weeks following FDA approval. Since launching in early April, Joenja® has 23 patients on paid therapy. We have received enrollments from significantly more patients who are in the process of payor reimbursement authorizations which have been proceeding as expected. The first sales of Joenja® will be booked and reported in the second quarter of 2023.

We anticipate providing updates on our commercial outlook for Joenja® in the second half of 2023.

RUCONEST® marketed for the treatment of acute HAE attacks

In the first quarter of 2023, RUCONEST® revenues were US$42.5 million, a 9% decline compared to the same quarter last year.

The decrease in overall revenues were primarily due to temporary disruptions in reimbursement for some patients on government insurance programs, which impacted the overall HAE market across all acute and prophylactic products. As a result, shipments to these patients were delayed and impacted February sales in particular. The HAE market disruptions seen in the first quarter have since resolved and we have seen a recovery in sales.

We are therefore maintaining our full-year outlook for low single digit growth in RUCONEST® revenues for 2023.

Underlying in-market demand for RUCONEST® continued to be strong, including a high number of new patient enrollments in the first quarter.

Strategic Highlights - regulatory and clinical updates

Leniolisib for APDS

Pharming made significant progress in the quarter towards our objective of obtaining regulatory approvals for leniolisib for APDS in additional key markets and for pediatric patients, and to identify additional indications for development of leniolisib beyond APDS.

EEA and U.K. market

In February 2023, Pharming announced that the European Medicines Agency's (EMA) Committee for Human Medicinal Products (CHMP) decided to shift its assessment of the Marketing Authorisation Application (MAA) for leniolisib to a standard review timetable. The list of questions received by Pharming from EMA included a request to submit updated data from the ongoing long-term extension study collected after the interim analysis included in the original MAA. As previously announced, we anticipate responding to the CHMP's list of questions in May 2023 and expect that the CHMP will issue its opinion on the leniolisib MAA in the second half of 2023. We anticipate that European marketing authorisation will follow approximately two months later.

In the U.K., and in line with the European Commission Decision Reliance Procedure (ECDRP), we intend to file the leniolisib dossier to the U.K.'s Medicines and Healthcare products Regulatory Agency (MHRA) within five days of a positive CHMP opinion; expected in the second half of 2023.

Japan

On March 9, 2023, we filed an Orphan Drug Designation (ODD) application with the Ministry of Health, Labor and Welfare (MHLW) in Japan. We expect to begin a clinical trial in Japan in second quarter of 2023 for APDS patients aged 12 and older. We currently anticipate marketing leniolisib in Japan directly following regulatory approval.

Pediatric clinical trial

In February 2023, as part of Pharming's Pediatric Investigational Plan (PIP), the Company announced that the first patient had been enrolled in its Phase III clinical trial evaluating the investigational drug leniolisib in children with APDS, at sites in the United States, Europe, and Japan. The single-arm, open-label, multinational clinical trial will evaluate the safety, tolerability, and efficacy of leniolisib in approximately 15 children aged 4 to 11 years who have a confirmed APDS diagnosis.

The second pediatric clinical trial for patients 1 to 6 years of age is scheduled to commence in the third quarter of 2023 and is also part of the PIP for leniolisib as a treatment for APDS in children.

Leniolisib for additional indications (PI3Kδ platform)

As announced in our Joenja® approval conference call on March 27, we have commenced working towards prioritizing other indications where leniolisib has the potential to deliver value for patients. PI3Kδ has been identified as an important factor in a variety of disease states, and leniolisib has demonstrated an attractive, long-term efficacy, safety and tolerability profile in clinical trials conducted in both healthy volunteers and patients. This provides a solid basis for our plans for the investigation and investment in further leniolisib indications. We are exploring further indications and will update the market later on this year.

Pre-Clinical Pipeline

OTL-105

We anticipate providing further updates as we move towards preparing an Investigational New Drug (IND) filing. If the data from pre-clinical HAE animal studies meet scientific requirements, then we expect to start IND-enabling pre-clinical studies in 2023.

Pompe

Based on our preclinical investigations and evaluations for potential differentiating features of the product candidate, it has been decided that the further development of this asset will be discontinued.

Other events during the quarter

On March 12 and 13, 2023, Pharming issued three press releases regarding the events surrounding Silicon Valley Bank. Pharming confirms that it has full access to its deposits.

Financial summary

Amounts in US $m except per share data	1Q 2023	1Q 2022
Consolidated Income Statement		
Revenues	**42.5**	**46.6**
Gross profit	**38.5**	**41.7**
Operating profit (loss)	**(13.7)**	**2.8**
Finance result, net	**(2.7)**	**1.8**
Income tax expense	**4.5**	**(0.8)**
Net Profit (loss)	**(12.2)**	**3.5**
Consolidated Balance Sheet		
Cash & marketable securities (Including restricted cash)	**186.2**	**190.7**
Share Information		
Basic earnings per share (US$)	**(0.019)**	**0.005**
Diluted earnings per share (US$)	**(0.017)**	**0.005**

Financial highlights

Total revenues for the first quarter of 2023 decreased by 9% to US$42.5 million compared to US$46.6 million in the first quarter of 2022. The decrease in overall revenues were primarily due to temporary disruptions in reimbursement for some government patients, which impacted the overall HAE market across all acute and prophylactic products and have since resolved. As a result, shipments to these patients were delayed and impacted February sales in particular. Underlying in-market demand for RUCONEST® however continued to be strong.

Gross profit decreased by 8% to US$38.5 million (1Q 2022: US$41.7 million), mainly due to the decrease in revenues.

Operating profit decreased to a loss of US$13.7 million versus an operating profit of US$2.8 million in the first quarter of 2022. This was mainly due to an expected increase in operating expenses from US$39.8 million in the first quarter of 2022 to US$52.7 million in the first quarter of this year. This increase was caused by a combination of launch preparation for leniolisib and increased payroll expenses due to business growth.

The Company had a net loss of US$12.2 million, versus a net profit of US$3.5 million in the first quarter of 2022. This was mainly due to the decrease in operating profit and more favorable currency exchange rate results in the first quarter of 2022 versus the first quarter of this year.

Negative cash flows from operations amounted to US$22.8 million in the first quarter of 2023. Cash and cash equivalents decreased by US$22.5 million to US$184.8 million from US$207.3 million at the end of the fourth quarter of 2022.

Outlook

For 2023, the Company anticipates:

- Continued low, single digit growth in annual revenues from RUCONEST®. Quarterly fluctuations are expected.
- Following FDA approval, we are commercializing Joenja® (leniolisib) in the U.S. commencing early April 2023.
- We anticipate a positive CHMP opinion for leniolisib in 2H 2023, Marketing Authorisation in Europe expected ~2 months later, followed by commercial launches in individual EU countries
- Following an anticipated positive CHMP opinion in 2H 2023, we intend to submit an ECDRP filing for leniolisib with the U.K. MHRA shortly thereafter. Approval expected several months later.
- Pharming will continue to allocate resources to accelerate future growth. Investments in launch preparations, commercialization and focused clinical developments for leniolisib including to support pediatric and Japanese approvals, as well as for the development of leniolisib in additional indications, will continue to impact profit throughout 2023. Our current cash on hand, including the continued cash flow of RUCONEST® are expected to be sufficient to fund these investments.
- Further details on our plans to develop leniolisib in additional indications to be provided in 2H 2023.
- Investments and continued focus on potential acquisitions and in-licensing of mid to late stage opportunities in rare diseases. Financing, if required, would come via a combination of our strong balance sheet and access to capital markets.

No further specific financial guidance for 2023 is provided.

Additional information

Presentation

The conference call presentation is available on the Pharming.com website from 07:30 CET, May 11.

Conference Call

The conference call will begin at 13:30 CET / 07:30 EDT on Thursday, May 11. A transcript of the call will be made available on the Pharming.com website the following day, Friday, May 12 from 16:30 CET.

Please note, the Company will only take questions from dial-in attendees.

Dial-in numbers for conference call

Netherlands: +31 85 888 7233
United Kingdom (Local): +44 20 3936 2999
United Kingdom (Toll-Free): +44 808 189 0158
United States (Local): +1 646 664 1960
United States (Toll-Free): +1 855 979 6654
Global Dial-In Numbers

Access code: 598957

Webcast Link: https://webcast.openbriefing.com/pharming1q23/

Financial Calendar 2023

Annual General Meeting of Shareholders May 17
2Q/1H 2023 financial results August 3
3Q 2023 financial results October 26

For further public information, contact:

Pharming Group N.V., Leiden, The Netherlands
Michael Levitan, VP Investor Relations & Corporate Communications
T: +1 (908) 705 1696

Heather Robertson, Investor Relations & Corporate Communications Manager
E: investor@pharming.com

FTI Consulting, London, UK
Victoria Foster Mitchell/Alex Shaw
T: +44 203 727 1000

LifeSpring Life Sciences Communication, Amsterdam, The Netherlands
Leon Melens
T: +31 6 53 81 64 27
E: pharming@lifespring.nl

About Pharming Group N.V.

Pharming Group N.V. (EURONEXT Amsterdam: PHARM/Nasdaq: PHAR) is a global biopharmaceutical company dedicated to transforming the lives of patients with rare, debilitating, and life-threatening diseases. Pharming is commercializing and developing an innovative portfolio of protein replacement therapies and precision medicines, including small molecules, biologics, and gene therapies that are in

early to late-stage development. Pharming is headquartered in Leiden, Netherlands, and has employees around the globe who serve patients in over 30 markets in North America, Europe, the Middle East, Africa, and Asia-Pacific.

For more information, visit www.pharming.com and find us on LinkedIn.

Forward-looking Statements

This press release 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 2022 Annual Report and the Annual Report on Form 20-F for the year ended December 31, 2022, 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 press release and are based on information available to Pharming as of the date of this 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

Inside Information

This press release relates to the disclosure of information that qualifies, or may have qualified, as inside information within the meaning of Article 7(1) of the EU Market Abuse Regulation.

Pharming Group N.V.

Condensed Consolidated Interim Financial Statements in US Dollars (unaudited)

For the period ended 31 March 2023

- Condensed consolidated statement of profit or loss
- Condensed consolidated statement of -comprehensive income
- Condensed consolidated balance sheet
- Condensed consolidated statement of cash flow

Condensed Consolidated Statement of Profit or Loss

For the period ended 31 March

Amounts in US$ '000	YTD 2023	YTD 2022
Revenues	**42,541**	**46,617**
Costs of sales	**(4,075)**	**(4,877)**
Gross profit	**38,466**	**41,740**
Other income	**579**	**873**
Research and development	(15,620)	(14,863)
General and administrative	(9,981)	(7,728)
Marketing and sales	(27,107)	(17,197)
Other Operating Costs	**(52,708)**	**(39,788)**
Operating profit (loss)	**(13,663)**	**2,825**
Other finance income	123	3,228
Other finance expenses	(2,795)	(1,379)
Finance result, net	**(2,672)**	**1,849**
Share of net profits in associates using the equity method	(339)	(441)
Profit (loss) before tax	**(16,674)**	**4,233**
Income tax expense	4,466	(772)
Profit (loss) for the year	**(12,208)**	**3,461**
Basic earnings per share (US$)	(0.019)	0.005
Diluted earnings per share (US$)	(0.017)	0.005

Condensed Consolidated Statement of Comprehensive Income

For the period ended 31 March

Amounts in US$ '000	YTD 2023	YTD 2022
Profit for the year	**(12,208)**	**3,461**
Currency translation differences	3,813	(3,216)
Fair value remeasurement investments	127	**(653)**
Items that may be subsequently reclassified to profit or loss	**3,940**	**(3,869)**
Other comprehensive income (loss), net of tax	**3,940**	**(3,869)**
Total comprehensive income for the year	**(8,268)**	**(408)**

Condensed Consolidated Balance Sheet

Amounts in US$ '000	March 31, 2023	December 31, 2022
Non-current assets		
Intangible assets	75,606	75,121
Property, plant and equipment	10,403	10,392
Right-of-use assets	30,369	28,753
Long-term prepayments	233	228
Deferred tax assets	29,350	22,973
Investment accounted for using the equity method	2,210	2,501
Investments in equity instruments designated as at FVTOCI	585	403
Investment in debt instruments designated as at FVTPL	6,972	6,827
Restricted cash	1,212	1,099
Total non-current assets	**156,940**	**148,297**
Current assets		
Inventories	48,127	42,326
Trade and other receivables	32,931	27,619
Restricted cash	218	213
Cash and cash equivalents	184,780	207,342
Total current assets	**266,056**	**277,500**
Total assets	**422,996**	**425,797**
Equity		
Share capital	7,518	7,509
Share premium	463,222	462,297
Other reserves	(4,906)	(8,737)
Accumulated deficit	(266,491)	(256,431)
Shareholders' equity	**199,343**	**204,638**
Non-current liabilities		
Convertible bonds	133,576	131,618
Lease liabilities	31,074	29,843
Total non-current liabilities	**164,650**	**161,461**
Current liabilities		
Convertible bonds	1,805	1,768
Trade and other payables	53,254	54,465
Lease liabilities	3,944	3,465
Total current liabilities	**59,003**	**59,698**
Total equity and liabilities	**422,996**	**425,797**

Condensed Consolidated Statement of Cash Flow

For the three months ended 31 March

Amounts in US$'000	YTD 2023	YTD 2022
Profit (loss) before tax	**(16,674)**	**4,233**
Non-cash adjustments:		
Depreciation, amortization, impairment of non-current assets	2,306	2,190
Equity settled share based payments	1,558	1,070
Other finance income	(123)	(3,228)
Other finance expenses	2,795	1,379
Share of net profits in associates using the equity method	339	441
Other	(455)	—
Operating cash flows before changes in working capital	**(10,254)**	**6,085**
Changes in working capital:		
Inventories	(5,801)	(2,297)
Trade and other receivables	(5,313)	(1,462)
Payables and other current liabilities	(1,211)	(1,645)
Restricted cash	117	(20)
Total changes in working capital	**(12,208)**	**(5,424)**
Interest received (paid)	117	(52)
Income taxes paid	(440)	—
Net cash flows generated from (used in) operating activities	**(22,785)**	**609**
Capital expenditure for property, plant and equipment	(215)	(208)
Investment intangible assets	—	(167)
Net cash flows used in investing activities	**(215)**	**(375)**
Payment of lease liabilities	(1,312)	(807)
Interests on loans	(2,013)	(2,100)
Proceeds of equity and warrants	695	18
Net cash flows generated from (used in) financing activities	**(2,630)**	**(2,889)**
Increase (decrease) of cash	**(25,630)**	**(2,655)**
Exchange rate effects	3,068	405
Cash and cash equivalents at 1 January	207,342	191,924
Total Cash and cash equivalents at 31 March	**184,780**	**189,674**