

# Pharming Group NV

Netherlands / Biotechnology  
 Primary exchange: Euronext Amsterdam /  
 Secondary exchange: Frankfurt  
 Bloomberg: PHARM NA  
 ISIN: NL0010391025

Q4/24 results/pipeline  
 expansion

**RATING**  
**BUY**

**PRICE TARGET**  
**€ 2.30**

Return Potential 214.0%  
 Risk Rating High

## STRONG Q4; TWO POTENTIAL BLOCKBUSTERS ADDED TO PIPELINE

On 13 March, the day it reported FY/24 results above guidance, Pharming announced the start of a second phase II trial of leniolisib for PIDs (primary immune deficiencies) with immune dysregulation beyond APDS (activated PI3K $\delta$  syndrome). The second trial, which began in February 2025, is for CVID (common variable immune deficiency) with immune dysregulation. The first trial, for PIDs with immune dysregulation linked to PI3K $\delta$  signalling, began in October 2024. The prevalence of PIDs linked to PI3K $\delta$  signalling and CVID is respectively 5x and 26x higher than for APDS, for which leniolisib is currently approved in the U.S. Both indications have blockbuster potential. In mid-March, Pharming also completed the acquisition of the Swedish company, Abliva, and its flagship drug candidate, KL1333. KL1333 is currently undergoing a pivotal trial (read-out expected in 2027) and is positioned to become the first standard of care in mitochondrial DNA-driven primary mitochondrial diseases (mtDNA). Annual revenue potential is also >USD1bn. Ahead of the acquisition of Abliva, we had expected 2025 operating costs to be close to the 2024 level. Management has indicated that the acquisition of Abliva will add USD30m to 2025 operating costs (of which USD17m for R&D and USD13m for one-off transaction/integration costs). Total incremental KL1333-related cost ahead of FDA approval expected in 2028 is ca. USD133m. The purchase price for Abliva was USD66.1m. KL1333 is a small molecule compound and is expected to generate a gross margin of over 95% on sales if approved. Given that KL1333 has blockbuster potential, the sum of the purchase price and subsequent transaction/integration and development costs looks very modest. We have adjusted our valuation model to reflect the strong Q4/24 results, the news on leniolisib for CVID and the completion of the Abliva acquisition. We now see fair value for the Pharming share at **€2.30** (previously: €1.70). We maintain our Buy recommendation. (p.t.o.)

### COMPANY PROFILE

Lead drug Ruconest, indicated for acute hereditary angioedema attacks, received EMA approval in 2010 and FDA approval in July 2014. Leniolisib, indicated for APDS, was approved by the FDA and launched in the U.S. in 2023. Pharming plans to expand the commercial availability of Leniolisib for APDS patients to key markets outside the U.S. and grow the drug's addressable patient population.

### MARKET DATA

As of 02 Apr 2025

Closing Price € 0.73  
 Shares outstanding 683.93m  
 Market Capitalisation € 500.98m  
 52-week Range € 0.65 / 1.01  
 Avg. Volume (12 Months) 5,292,864

Multiples	2024	2025E	2026E
P/E	n.a.	n.a.	n.a.
EV/Sales	1.7	1.6	1.4
EV/EBIT	n.a.	n.a.	n.a.
Div. Yield	0.0%	0.0%	0.0%

### STOCK OVERVIEW



### FINANCIAL HISTORY & PROJECTIONS

	2022	2023	2024	2025E	2026E	2027E
Revenue (\$ m)	205.6	245.3	297.2	325.0	368.3	431.8
Y-o-y growth	3.4%	19.3%	21.1%	9.4%	13.3%	17.2%
EBIT (\$ m)	18.2	-5.4	-8.6	-9.5	-38.8	30.2
EBIT margin	8.9%	-2.2%	-2.9%	-2.9%	-10.5%	7.0%
Net income (\$ m)	13.7	-10.5	-11.0	-11.1	-40.5	20.9
EPS (diluted) (\$)	1.93	-1.60	-1.50	-1.51	-5.52	2.84
DPS (\$)	0.00	0.00	0.00	0.00	0.00	0.00
FCF (\$m)	20.5	-18.8	-0.5	11.4	-38.6	20.6
Net gearing	-20.5%	-19.8%	-25.7%	-31.0%	-14.2%	-21.4%
Liquid assets (\$ m)	207.3	213.4	167.9	179.3	140.6	161.2

### RISKS

The main risks to our price target include slower sales growth for Ruconest and Leniolisib than we currently model.

### COMPANY DATA

As of 31 Dec 2024

Liquid Assets \$ 167.89m  
 Current Assets \$ 278.70m  
 Intangible Assets \$ 61.04m  
 Total Assets \$ 400.79m  
 Current Liabilities \$ 73.80m  
 Shareholders' Equity \$ 221.86m

### SHAREHOLDERS

BlackRock Institutional Trust Co., N.A. 2.8%  
 Morgan Stanley Inv. Man. Ltd. (UK) 2.8%  
 DWS Investment GmbH 2.2%  
 JP Morgan Asset Management 1.4%  
 Free float and other 90.8%



**Q4/24 Ruconest and leniolisib sales both above our forecast** Q4/24 sales of USD92.7m (FBe: USD83.0m; Q4/23: USD81.2m) rose 14.1% and were 11.7% above our forecast. The full-year sales number of USD297.2m was also above company guidance of USD280m-USD295m. Strong underlying demand pushed Q4/24 Ruconest sales up 8.6% to USD79.6m (FBe: USD70.9m; Q4/23: USD73.3m) - USD8.7m above our expectation. Meanwhile Q4/24 leniolisib sales were USD1m better than our forecast.

**Figure 1: Recent quarterly sales and profit development and FY/25 forecast**

USDk	Q4 23	2023A	Q1 24	Q2 24	Q3 24	Q4 24E	Q4 24A	2024E	2024A	2025E
<b>Sales</b>	<b>81,217</b>	<b>245,316</b>	<b>55,586</b>	<b>74,093</b>	<b>74,849</b>	<b>82,972</b>	<b>92,672</b>	<b>287,500</b>	<b>297,200</b>	<b>325,000</b>
<b>yoy change</b>	<b>48.7%</b>	<b>19.3%</b>	<b>30.7%</b>	<b>35.0%</b>	<b>12.3%</b>	<b>2.2%</b>	<b>14.1%</b>	<b>17.2%</b>	<b>21.1%</b>	<b>9.4%</b>
of which:										
Ruconest	73,288	227,134	46,000	62,973	63,649	70,896	79,578	243,518	252,200	271,150
yoy chng (%)	34.2%	10.5%	8.1%	23.2%	5.7%	-4.0%	8.6%	7.2%	11.0%	7.5%
Leniolisib	7,890	18,182	9,600	11,106	11,200	12,076	13,094	43,982	45,000	53,850
chng (%)	n.a.	n.a.	n.a.	192.9%	72.3%	53.1%	66.0%	141.9%	147.5%	19.7%
<b>Gross profit</b>	<b>74,099</b>	<b>220,104</b>	<b>47,200</b>	<b>66,112</b>	<b>68,030</b>	<b>73,845</b>	<b>80,459</b>	<b>255,187</b>	<b>261,801</b>	<b>295,734</b>
<b>margin %</b>	<b>91.2%</b>	<b>89.7%</b>	<b>84.9%</b>	<b>89.2%</b>	<b>90.9%</b>	<b>89.0%</b>	<b>86.8%</b>	<b>88.8%</b>	<b>88.1%</b>	<b>91.0%</b>
Other income	538	23,349	345	912	777	500	143	2,534	2,177	2,000
% sales	0.7%	9.5%	0.6%	1.2%	1.0%	0.6%	0.2%	0.9%	0.7%	0.6%
R&D	-11,627	-68,914	-18,521	-21,597	-20,721	-21,000	-22,322	-81,839	-83,161	-96,200
% sales	14.3%	28.1%	33.3%	29.1%	27.7%	25.3%	24.1%	28.5%	28.0%	29.6%
G&A	-24,028	-55,877	-15,087	-15,620	-15,292	-16,200	-24,620	-62,199	-70,619	-77,000
% sales	29.6%	22.8%	27.1%	21.1%	20.4%	19.5%	26.6%	21.6%	23.8%	23.7%
Marketing	-37,913	-124,049	-30,249	-32,928	-28,686	-31,500	-26,956	-123,363	-118,819	-134,000
% sales	46.7%	50.6%	54.4%	44.4%	38.3%	38.0%	29.1%	42.9%	40.0%	41.2%
Other op. costs	-73,568	-248,840	-63,857	-70,145	-64,699	-68,700	-73,898	-267,401	-272,599	-307,200
% sales	90.6%	101.4%	114.9%	94.7%	86.4%	82.8%	79.7%	93.0%	91.7%	94.5%
<b>EBIT</b>	<b>1,069</b>	<b>-5,387</b>	<b>-16,312</b>	<b>-3,121</b>	<b>4,108</b>	<b>5,645</b>	<b>6,704</b>	<b>-9,680</b>	<b>-8,621</b>	<b>-9,466</b>
<b>margin %</b>	<b>1.3%</b>	<b>-2.2%</b>	<b>-29.3%</b>	<b>-4.2%</b>	<b>5.5%</b>	<b>6.8%</b>	<b>7.2%</b>	<b>-3.4%</b>	<b>-2.9%</b>	<b>-2.9%</b>

Source: First Berlin Equity Research estimates, Pharming Group NV

**EBIT back in the black in the final two quarters of 2024** FY/24 EBIT was again in the red at USD-8.6m (FBe: USD-9.7m, FY/23: USD-5.4m) as Pharming continued to invest in marketing of leniolisib in the U.S., preparation for the launch of leniolisib outside the U.S., and R&D to expand leniolisib's addressable patient population. However, increasing sales and moderating costs meant that EBIT was positive in both Q3/24 and Q4/24, reaching USD6.7m in the final quarter.

**Management expects Ruconest sales to grow at high single digit rate in 2025**

Pharming is guiding towards sales of USD315m-USD335m for FY/25, implying sales growth of 6.0%-12.7%. Management indicated during the analysts' call that Ruconest is expected to grow at a high-single digit percentage clip this year despite the likely launch at mid-year of an orally administered competing drug in Ruconest's on-demand segment of the hereditary angioedema market.

Competition to Ruconest has increased significantly in recent years. However, excellent efficacy has ensured the resilience of Ruconest's sales, which have grown 33% since 2019. The competitive landscape for hereditary angioedema (HAE) therapies has been very dynamic in recent years. The two main trends have been market share gains by prophylactic and/or orally administered products such as Takhzyro and Orladeyo from on-demand products, such as Ruconest, which are used to stop attacks once they have already started. However, Ruconest sales have had only one down year (2021) since the product's launch in the EU and US in 2010 and 2014 respectively.

**Figure 2: Recent sales development of Ruconest and competitors (USDm)**

Drug	Company	2018	2019	2020	2021	2022	2023	2024
Berinert	CSL Behring	n.a.	n.a.	n.a.	n.a.	288	248	n.a.
% Δ		n.a.	n.a.	n.a.	n.a.	n.a.	-13.9%	n.a.
Cinryze	Takeda	28	224	213	172	147	124	114
% Δ		n.a.	701.0%	-4.9%	-19.3%	-14.4%	-15.6%	-8.1%
Firazyr	Takeda	58	300	261	238	189	161	119
% Δ		n.a.	420.5%	-13.1%	-9.0%	-20.4%	-14.8%	-26.1%
Generic Icatibant	several	n.a.	n.a.	n.a.	n.a.	185	197	n.a.
% Δ		n.a.	n.a.	n.a.	n.a.	n.a.	6.5%	n.a.
Haegarda	CSL Behring	n.a.	n.a.	n.a.	n.a.	436	457	n.a.
% Δ		n.a.	n.a.	n.a.	n.a.	n.a.	4.8%	n.a.
Kalbitor	Takeda	11	42	38	40	30	30	n.a.
% Δ		n.a.	286.2%	-8.8%	5.9%	-25.7%	0.0%	n.a.
Orladeyo	BioCryst	0	0	0	123	252	326	438
% Δ		n.a.	n.a.	n.a.	n.a.	105.3%	29.6%	34.4%
Ruconest	Pharming	160	189	212	199	206	227	252
% Δ		n.a.	18.5%	12.2%	-6.3%	3.4%	10.5%	11.0%
Takhzyro	Takeda	87	628	844	919	1,144	1,254	1,490
% Δ		n.a.	617.9%	34.5%	8.8%	24.5%	9.6%	18.8%

Source: companies; First Berlin Equity Research

**Resilience of Ruconest sales based on efficacy...** In our view there are two reasons for the resilience of Ruconest's sales. Firstly, the drug has a high level of efficacy. In its most common forms, HAE is caused by a functional deficiency of a plasma protein called C1-inhibitor. Ruconest is a recombinant C1-inhibitor protein replacement therapy and so tackles the root cause of HAE. As it is intravenously delivered, it is immediately and completely bioavailable to stop the progression of HAE attacks. Results of an investigator-initiated comparative real-world study of therapies for acute attacks of HAE published by Pharming in December 2018 showed a significantly lower re-dosing rate for Ruconest than for Firazyr. 18 (90%) of 20 attacks treated with Ruconest were resolved after the first dose. According to Pharming this number would probably have been 100% had two patients not underdosed themselves by using only 1 vial of 2,100 IU compared with the 50 IU/kg dose recommended on the label. By contrast 11 (44%) of the 25 patients who took Firazyr required a second dose.

**...and treatment of breakthrough attacks suffered by patients using prophylactic therapies** Secondly, studies indicate that 50% of HAE patients using leading prophylactic therapies Haegarda and Takhzyro suffer breakthrough attacks. For Orladeyo this figure is 90%. HAE patients using prophylactic therapies typically use on-demand treatments such as Ruconest to halt breakthrough attacks.

**Figure 3: HAE therapies in clinical development**

Company	Asset	Mode of Action	Route of Administration	Trial Phase	Role in Therapy
KalVista	Sebetralstat	Kallikrein inhibitor	Oral	PDUFA date: 17/06/25	On demand
Pharvaris	Deucricitabant (PHVS416/PHVS719)	B2 receptor antagonist	Oral	3	On demand and prophylaxis
CSL Behring	Garadacimab	Anti-factor XII mAb	Subcutaneous	Filed (approved outside U.S.)	Prophylaxis
Ionis	Donidalorsen	Prekallikrein inhibitor	Subcutaneous	PDUFA date: 21/08/25	Prophylaxis
Astria	Navenibart	Kallikrein inhibitor	Subcutaneous	2/3	Prophylaxis
ADARx	ADX-324	siRNA	Subcutaneous	1	Prophylaxis
Intellia	NTLA-2002	Gene therapy	IV	3	Functional cure

Source: companies

**We view the threat to Ruconest from Kalvista's sebetralstat as limited** The latest threat to Ruconest's position emanates from Kalvista's sebetralstat, which is the first oral on-demand therapy candidate for HAE. Subject to FDA approval, sebetralstat could be launched in the U.S. in June 2025.

However, sebetralstat was tested in a patient population that is generally responsive to Firazyr (icatibant) and its generic counterpart. A key part of the client base for Ruconest is comprised of patients who have failed on icatibant, which only serves the bradykinin/kallikrein pathway rather than addressing the root cause of HAE.

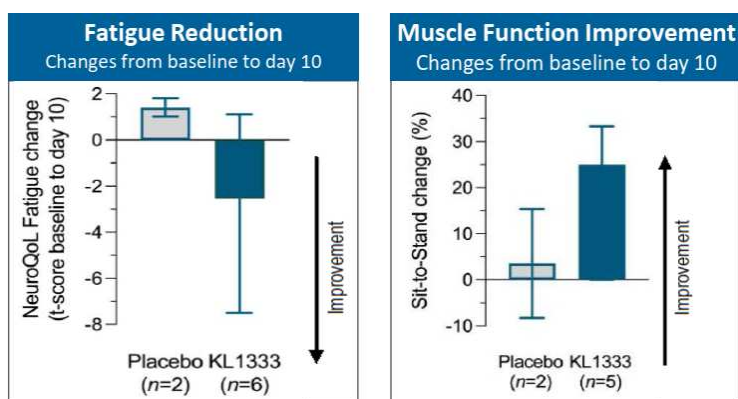
**KL1333 has blockbuster potential** KL1333 is currently undergoing a pivotal trial (read-out expected in 2027) and is positioned to become the first standard of care in mitochondrial DNA-driven primary mitochondrial diseases (mtDNA). Annual revenue potential is >USD1bn. Mitochondrial diseases are a group of genetic disorders characterised by dysfunctional mitochondria due to mutations in mitochondrial DNA (mtDNA) or nuclear DNA (nDNA). According to Gorman et al (2015) the prevalence of disease caused by pathogenic mutations of mitochondrial DNA at ca. one in 5,000 people is ca. 7x higher than disease caused by pathogenic mutations of nDNA.

Mitochondria are intracellular "powerhouses" that produce adenosine triphosphate (ATP) and carry out diverse functions for cellular energy metabolism. ATP provides energy to drive and support many processes in living cells, such as muscle contraction, nerve impulse propagation, and chemical synthesis. The maintenance of an optimal NAD<sup>+</sup>/NADH (nicotinamide adenine dinucleotide<sup>+</sup>/ nicotinamide adenine dinucleotide + hydrogen) ratio is essential for the production of ATP. This ratio becomes abnormal in mitochondrial disease. Patients suffer from severe fatigue, myopathy (muscle weakness) and reduced life expectancy.

**Phase 1b study showed KL1333 reduces mtDNA patients' fatigue and myopathy**

KL1333 reacts with NAD(P)H:quinone oxidoreductase 1 (NQO1) as a substrate, resulting in increases in intracellular NAD<sup>+</sup> levels via NADH oxidation and normalising the NAD<sup>+</sup>/NADH ratio. A placebo-controlled phase 1b study conducted between 2019 and 2021 demonstrated that KL1333 dosed at 50mg/day reduced patients' fatigue and myopathy after only 10 days (see figure 4).

**Figure 4: KL1333 reduced patients' fatigue and myopathy after only 10 days, 50mg/day**



Source: Pharming, Pizzamiglio et al (2025)

**First cohort of KL1333 phase 2 showed both primary endpoints having passed futility**

FALCON, a placebo-controlled phase 2 study of KL1333, began in December 2022. Given that mitochondrial disease is an orphan indication, both the FDA and EMA have agreed that approval can be granted in the case of a positive trial outcome. Falcon's two primary endpoints are: 1) fatigue using the PROMIS Fatigue Mitochondrial Disease Short Form, 2) muscle weakness using the 30 second Sit-to-Stand test. These are alternate primary endpoints, i.e. success is required in only one of the two is required for approval. The FALCON study is comprised of two cohorts, WAVE 1 (n=40) and WAVE 2 (n=140).



Interim analysis of WAVE 1 patients at 24 weeks conducted in Q3/24 showed both primary endpoints having passed futility (in the context of clinical research, futility is often used to indicate that a trial is unlikely to meet its original goal, e.g. demonstrating treatment efficacy). Read-out of WAVE 2 is anticipated in 2027.

**KL1333 is the only mtDNA-related mitochondrial disease drug candidate to address the full scope of the disease** KL1333 is positioned to become the first standard of care in mtDNA-related mitochondrial disease. In our view, the most important point about the competitive landscape for the development of drugs for mitochondrial diseases is that KL1333 is the only one of the drug candidates in figure 5 to address the wider mtDNA market. MELAS (Mitochondrial Encephalopathy, Lactic Acidosis, and Stroke-like episodes) and Barth syndrome are smaller and very small subsets of the mtDNA market respectively.

**Competitors' MELAS/Barth focus is a small subset of overall mtDNA market** MELAS is a multisystemic mitochondrial disease that was first identified as a clinical entity in 1984. Symptoms can vary between individuals but typically include recurrent stroke-like episodes, encephalopathy and the accumulation of lactic acid in the bloodstream. While data on disease incidence are limited, Hueng-Chuen Fan et al (2021) cite studies which estimated the prevalence of MELAS syndrome at 0.18:100,000 in Japan, 1.41:100,000 in the north east of England, 2:100,000 in Sweden, 18.4:100,000 in Finland, and 236:100,000 in Australia. With the exception of the outlier figure for Australia, these figures are below, mostly very far below, the estimated prevalence of mtDNA-related mitochondrial disease at 20:100,000 individuals. As figure 5 shows, the number of Barth patients is estimated at 150 globally.

**Figure 5: Competitive landscape in mtDNA**

Company	Asset	Asset type	Mode of Action	Stage/Route of Administration	Patient Group Focus	Commentary
Pharming	KL1333	Small organic molecule	NAD <sup>+</sup> /NADH modulation	Pivotal Oral	mtDNA mutations (mtDNA deletion, m.8344A>G, MELAS-MIDD)	Ongoing potentially registrational phase 2 trial evaluating alternative independent primary endpoints fatigue and myopathy
Khondrion	Sonlicromanol	ROS-redox modulator	Oxidative stress modulator	Upcoming phase 3 Oral	MELAS	Focus on m.3243A>G patients (primarily MELAS)
Stealth Biotherapeutics	Elamipretide	Tetrapeptide	Mitochondrial cardiolipin-binding peptide	Pivotal Subcutaneous	Subgroup of mitochondrial myopathy patients (nDNA mutations)	PDUFA date for Elamipretide for ultra-rare Barth Syndrome (150 patients globally) 29 April 2025
Tisento Therapeutics	Zagociguat	Small Molecule	Guanylate cyclase (sGC) stimulator	Ongoing phase 2b Oral	MELAS	Open-label MELAS phase 2a completed. Phase 2b trial with focus on fatigue, myopathy and cognition ongoing

Source: Pharming

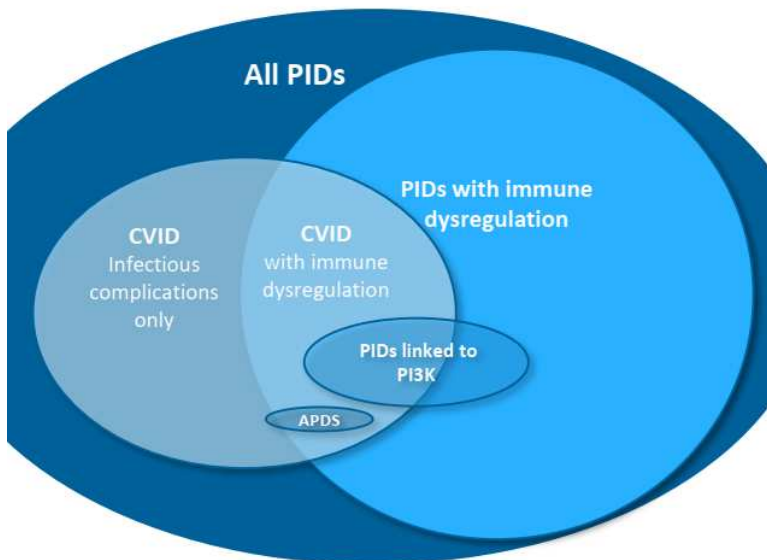
**Leniolisib approved in the U.S. for APDS patients ≥12 years in March 2023** Pharming's leniolisib was approved in the U.S. for APDS patients 12 years and older in March 2023. APDS is a PID which is caused by a mutation in the PIK3CD or PIK3R1 genes that results in immune dysregulation due to an increase of activity of phosphoinositide-3-kinase delta, a promoter of activity in the immune system. APDS has an estimated prevalence of 1 to 2 per million. Individuals suffering from APDS often have lymphoproliferation. Lymphoproliferation refers to excessive production of lymphocytes. The most important types of lymphocytes are the white blood cells, B cells and T cells. APDS patients have poorly functioning B cells and T cells. Beginning in childhood, people with APDS develop recurrent infections, particularly in the lungs, sinuses, and ears. Over time, recurrent respiratory tract infections can lead to a condition called bronchiectasis, leading to serious breathing problems. People with APDS may also suffer from chronic active viral infections, for example EpsteinBarr virus or cytomegalovirus infections. Sufferers also frequently develop lymphomas and other types of tumors. Leniolisib inhibits the production of phosphatidylinositol-3-4-5-trisphosphate, which serves as an important cellular messenger and regulates a multitude of cell functions such as proliferation, differentiation, cytokine production, cell survival, angiogenesis, and metabolism.



**Phase 2 PoC trial of leniolisib for PI3K-linked PIDs began last October** In October last year Pharming announced the start of a phase 2 proof of concept trial of leniolisib for other PIDs characterised by immune dysregulation linked to PI3K signalling in lymphocytes. The genes involved include ALPS FAS, CTLA4, NFKB1 and PTEN and the diseases can be described as ALPS-FAS, CTLA4 haploinsufficiency, NFKB1 haploinsufficiency and PTEN deficiency. Study completion is expected in late 2025. One of the aims of the trial is to pick the best dose regimen for a phase 3 trial. Assuming the start of a phase 3 trial in 2026, we expect approval and launch in 2029. In terms of market potential, we note that the estimated combined prevalence of these PIDs at 7.5 per million is 5x that of the 1-2 per million for APDS.

**Phase 2 PoC trial of leniolisib for CVIDs began in March 2025** On 20 March Pharming announced the start of a further phase 2 proof of concept trial of leniolisib - this time for CVID with immune dysregulation. Study completion is expected in late 2026. The trial has been designed to inform a subsequent phase 3 programme. Assuming the start of a phase 3 trial in 2027, we expect approval and launch in 2030. CVID is the most prevalent symptomatic PID, accounting for more than 50% of cases of the disease. For most CVID patients, the exact causes remain unclear. Unlike APDS and PIDs linked to PI3K, which both have genetic drivers, genetic differences explain only around 20% of CVID cases. CVID is thought to have both genetic and environmental causes. The variable aspect of CVID refers to the approximately half of patients who display autoimmune, lymphoproliferative and/or end-organ lympho-infiltrative clinical manifestations driven by immune dysregulation which result in non-infectious complications in addition to heightened susceptibility to infection. These non-infectious complications include autoimmune, gastrointestinal, pulmonary, lymphoproliferative, and malignant complications. The global prevalence of the targeted CVID with immune dysregulation population is approximately 39 patients per million.

**Figure 6: Estimated prevalence of APDS, PIDs linked to PI3K, CVID with immune dysregulation\***



\*Not to scale with population sizes

Prevalence estimates	
APDS	1.5/million
PIDs linked to PI3K	7.5/million
CVID with immune dysregulation	39/million*

\*CVID prevalence excludes APDS but includes most of the PIDs linked to the PI3K patient population

Source: Pharming NV



### CVID patients with immune dysregulation poorly served by immunoglobulin replacement therapy SoC

Immunoglobulin replacement therapy is the standard of care for CVID. Since the widespread adoption of this treatment, mortality of patients with CVID decreased from 30% in the early 1990s to 15% in the early 2000s, in a cohort of 240 patients in the United Kingdom and 334 patients from the European Society for Immunodeficiencies registry, respectively. All patients were followed for approximately two decades. The improved survival in CVID patients has been attributed to the reduction of infectious complications thanks to the widespread use of immunoglobulin replacement and improved anti-microbial therapies (9–12). While overall survival has improved, CVID patients with immune dysregulation have an unmet medical need with an 11-fold enhanced rate of mortality compared to CVID patients with infectious manifestations alone, and the majority exhibit a spectrum of clinical manifestations with similarities to APDS patients.

**Figure 7: Changes to our forecasts**

All figures in USD '000	2025E			2026E			2027E		
	new	old	% Δ	new	old	% Δ	new	old	% Δ
<b>Revenues</b>	<b>325,000</b>	<b>309,632</b>	<b>5.0%</b>	<b>368,277</b>	<b>338,878</b>	<b>8.7%</b>	<b>431,761</b>	<b>413,482</b>	<b>4.4%</b>
of which:									
Ruconest	271,150	243,518	11.3%	257,593	231,342	11.3%	275,624	254,476	8.3%
Leniolisib	53,850	66,115	-18.6%	110,685	107,537	2.9%	156,137	159,006	-1.8%
Costs of sales	29,266	34,376	-14.9%	36,928	39,500	-6.5%	43,257	54,701	-20.9%
<b>Gross profit</b>	<b>295,734</b>	<b>275,256</b>	<b>7.4%</b>	<b>331,349</b>	<b>299,378</b>	<b>10.7%</b>	<b>388,504</b>	<b>358,781</b>	<b>8.3%</b>
Other income	2,000	2,000	0.0%	2,100	2,100	0.0%	2,153	2,153	0.0%
Research and development cost	96,200	82,053	17.2%	147,311	81,331	81.1%	116,575	82,696	41.0%
General and administrative cost	77,000	63,475	21.3%	71,814	64,387	11.5%	73,399	70,292	4.4%
Marketing and sales cost	129,000	130,045	-0.8%	138,104	138,940	-0.6%	155,434	146,786	5.9%
Milestones/PRV income/(expense)	-5,000	-5,000	0.0%	-15,000	-15,000	n.a.	-15,000	-15,000	n.a.
<b>Operating income (EBIT)</b>	<b>-9,466</b>	<b>-3,316</b>	<b>n.a.</b>	<b>-38,780</b>	<b>1,821</b>	<b>n.a.</b>	<b>30,248</b>	<b>46,159</b>	<b>-34.5%</b>
Net financial income/(expense)	-1,646	-2,056	n.a.	-1,738	-2,433	n.a.	-2,144	-2,424	n.a.
Income/(expense) from associates	0	0	n.a.	0	0	n.a.	0	0	n.a.
<b>Pre-tax income (EBT)</b>	<b>-11,112</b>	<b>-5,372</b>	<b>n.a.</b>	<b>-40,518</b>	<b>-612</b>	<b>n.a.</b>	<b>28,104</b>	<b>43,735</b>	<b>-35.7%</b>
Income tax credit/(expense)	0	645	n.a.	0	158	n.a.	-7,251	-11,284	n.a.
<b>Net income/(loss)</b>	<b>-11,112</b>	<b>-4,727</b>	<b>n.a.</b>	<b>-40,518</b>	<b>-454</b>	<b>n.a.</b>	<b>20,853</b>	<b>32,451</b>	<b>-35.7%</b>
<b>Diluted EPS (US cents)</b>	<b>-1.51</b>	<b>-0.64</b>	<b>n.a.</b>	<b>-5.52</b>	<b>-0.06</b>	<b>n.a.</b>	<b>2.84</b>	<b>4.42</b>	<b>-35.7%</b>

Source: First Berlin Equity Research estimates

**Buy recommendation maintained, price target raised from €1.70 to €2.30** Pharming is guiding towards sales of USD315m - USD335m for 2025. We assume the midpoint of this range, i.e. USD325m. As stated above, management indicated during the Q4/24 analysts' call that Ruconest is expected to grow at a high-single digit percentage rate this year. We assume growth of 7.5% to take 2025 Ruconest sales to USD271.2m (2024: USD252.2m). We model a decline of 5.0% in Ruconest sales in 2026 as patients try sebetralstat. However, we expect a substantial number of these patients to return to Ruconest as they discover that their needs are better served by the Pharming drug. We therefore expect Ruconest sales to rebound 7% in 2027.

Leniolisib accounts for the USD53.9m balance of our 2025 sales forecast. This figure is 18.6% below our previous forecast of USD66.2m but still implies 19.7% growth compared with 2024. We expect leniolisib sales to accelerate in H2/25 as screening for variants of uncertain significance in the PIK3CD or PIK3R1 genes boosts the number of patients on paid therapy.



We have further adjusted our P&L forecast to take account of the total USD120m incremental KL1333-related cost ahead of the drug candidate's expected FDA approval in 2028. We maintain our Buy recommendation and raise the price target from €1.70 to €2.30.

**Figure 8: Valuation model**

Compound	Indication	Present Value	Patient Pop	Treatment Cost	Market Size	Market Share	Peak Sales	Gross margin	Discount Factor	Patent Life <sup>2)</sup>	Time to Market
Ruconest (US)	HAE-AA	€1,296.9M	4,000	€ 297,273	€2,081M	10%	€426M	92%	12%	12	-
Ruconest (ROW)	HAE-AA	€0.9M	8,000	€ 90,909	€727M	1%	€9M	33%	12%	16	-
Leniolisib (US)	APDS	€485.1M	320	€ 540,000	€173M	100%	€140M	93%	10%	14	-
Leniolisib (ROW)	APDS	€317.0M	550	€ 324,000	€178M	100%	€10M	83%	10%	11	1 year
Leniolisib (US)	PI3K $\delta$ -linked PIDS	€150.7M	1,600	€ 540,000	€864M	100%	€743M	82%	4%	8	4 years
Leniolisib (ROW)	PI3K $\delta$ -linked PIDS	€81.1M	2,750	€ 324,000	€891M	100%	€531M	80%	14%	8	4 years
Leniolisib (US)	CVID	€438.2M	4,800	€ 540,000	€2,592M	100%	€2,201M	82%	14%	7	5 years
Leniolisib (ROW)	CVID	€199.8M	8,250	€ 324,000	€2,673M	100%	€1,753M	80%	14%	7	5 years
KL1333 (US)	mtDNA-related MD	€800.5M	13,500	€ 172,727	€2332M	50%	€991M	82%	12%	6	4 years
KL1333 (ROW)	mtDNA-related MD	€261.2M	12,500	€ 68,182	€52M	50%	€362M	82%	12%	6	4 years
<b>PV of gross profits after mktg cost</b>		<b>€4,031.4M</b>									
Costs PV		€2,123.9M									
<b>PV after costs</b>		<b>€1,907.4M</b>									
Leniolisib/KL1333 milestones		€224.9M									
Net cash (pro-forma)		€127.3M									
Fair Value		€1,809.8M									
Share Count (fully diluted, PV)		786,571K									
<b>Fair value per share</b>		<b>€ 2.30</b>									

1) A project typically refers to a specific indication or, where necessary or relevant, a combination between indication and geographic market

2) Remaining patent life in years after point of approval

Source: First Berlin Equity Research estimates

**Figure 9: Changes to our valuation model**

	New	Old	Delta
<b>PV of gross profit after marketing cost</b>	<b>€4,031.4M</b>	<b>€3,022.6M</b>	<b>33.4%</b>
Costs PV	€2,123.9M	€1,755.5M	21.0%
<b>PV after costs</b>	<b>€1,907.4M</b>	<b>€1,267.1M</b>	<b>50.5%</b>
Leniolisib/KL1333 milestones	€224.9M	€94.7M	137.5%
Proforma net cash	€127.3M	€110.4M	15.3%
Fair Value	€1,809.8M	€1,282.8M	41.1%
Share Count (fully diluted, PV)	786,572K	768,304K	2.4%
<b>Fair value per share</b>	<b>€ 2.30</b>	<b>€ 1.67</b>	<b>37.8%</b>

Source: First Berlin Equity Research estimates





## INCOME STATEMENT

All figures in USD '000	2022A	2023A	2024A	2025E	2026E	2027E
<b>Revenues</b>	<b>205,622</b>	<b>245,316</b>	<b>297,200</b>	<b>325,000</b>	<b>368,277</b>	<b>431,761</b>
Costs of sales	-17,562	-25,212	-35,399	-29,266	-36,928	-43,257
<b>Gross profit</b>	<b>188,060</b>	<b>220,104</b>	<b>261,801</b>	<b>295,734</b>	<b>331,349</b>	<b>388,504</b>
Other income	14,523	23,349	2,177	2,000	2,100	2,153
Research and development	-52,531	-68,914	-83,161	-96,200	-147,311	-116,575
General and administrative	-46,016	-55,877	-70,619	-77,000	-71,814	-73,399
Marketing and sales	-85,803	-124,049	-118,819	-129,000	-138,104	-155,434
Milestones/PRV sales	0	0	0	-5,000	-15,000	-15,000
<b>Operating income (EBIT)</b>	<b>18,233</b>	<b>-5,387</b>	<b>-8,621</b>	<b>-9,466</b>	<b>-38,780</b>	<b>30,248</b>
Net financial result	-2,163	-6,336	1,866	-1,646	-1,738	-2,144
Associates	-1,083	-289	-1,758	0	0	0
<b>Pre-tax income (EBT)</b>	<b>14,987</b>	<b>-12,012</b>	<b>-8,513</b>	<b>-11,112</b>	<b>-40,518</b>	<b>28,104</b>
Income taxes	-1,313	1,464	-2,514	0	0	-7,251
<b>Net income / loss</b>	<b>13,674</b>	<b>-10,548</b>	<b>-11,027</b>	<b>-11,112</b>	<b>-40,518</b>	<b>20,854</b>
<b>Diluted EPS (US cents)</b>	<b>1.934</b>	<b>-1.600</b>	<b>-1.500</b>	<b>-1.514</b>	<b>-5.522</b>	<b>2.842</b>
<b>EBITDA</b>	<b>26,753</b>	<b>2,708</b>	<b>-597</b>	<b>284</b>	<b>-27,732</b>	<b>43,200</b>
<b>Ratios</b>						
Gross margin on revenues	91.5%	89.7%	88.1%	91.0%	90.0%	90.0%
EBITDA margin on revenues	13.0%	1.1%	n.m.	0.1%	n.m.	10.0%
EBIT margin on revenues	8.9%	n.m.	n.m.	n.m.	n.m.	7.0%
Net margin on revenues	6.7%	n.m.	n.m.	n.m.	n.m.	4.8%
<b>Expenses as % of revenues</b>						
Cost of sales	8.5%	10.3%	11.9%	9.0%	10.0%	10.0%
Research and development	25.5%	28.1%	28.0%	29.6%	40.0%	27.0%
General and administrative	22.4%	22.8%	23.8%	23.7%	19.5%	17.0%
Marketing and sales	41.7%	50.6%	40.0%	39.7%	37.5%	36.0%
<b>Y-Y Growth</b>						
Revenues	3.4%	19.3%	21.1%	9.4%	13.3%	17.2%
Operating income	34.5%	n.m.	n.m.	n.m.	n.m.	n.m.
Net income/ loss	-14.5%	n.m.	n.m.	n.m.	n.m.	n.m.



## BALANCE SHEET

All figures in USD '000	2022A	2023A	2024A	2025E	2026E	2027E
<b>Assets</b>						
<b>Current assets, total</b>	<b>277,500</b>	<b>316,342</b>	<b>278,696</b>	<b>289,815</b>	<b>265,912</b>	<b>308,072</b>
Cash and cash equivalents	207,342	61,741	54,944	66,313	27,688	48,254
Marketable securities	0	151,683	112,949	112,949	112,949	112,949
Restricted cash	213	0	0	0	0	0
Receivables	27,619	46,158	55,079	43,654	49,467	57,994
Inventories	42,326	56,760	55,724	66,899	75,808	88,875
<b>Non-current assets, total</b>	<b>148,297</b>	<b>146,512</b>	<b>122,091</b>	<b>122,452</b>	<b>119,898</b>	<b>119,215</b>
Property, plant & equipment	10,392	9,689	7,752	9,750	10,312	12,089
Right of use assets	28,753	23,777	16,382	19,500	22,097	25,906
Long term prepayments	228	92	90	360	408	479
Deferred tax assets	22,973	29,761	31,090	31,090	31,090	31,090
Investments accounted for using the equity method	2,501	2,285	466	466	466	466
Investments in FVTOCI equity instruments	403	2,020	0	0	0	0
Investments in FVTPL debt instruments	6,827	6,093	3,767	3,767	3,767	3,767
Goodwill & other intangibles	75,121	71,267	61,039	56,014	50,253	43,913
Restricted cash	1,099	1,528	1,505	1,505	1,505	1,505
<b>Total assets</b>	<b>425,797</b>	<b>462,854</b>	<b>400,787</b>	<b>412,268</b>	<b>385,810</b>	<b>427,287</b>
<b>Shareholders' equity &amp; debt</b>						
<b>Current liabilities, total</b>	<b>59,698</b>	<b>77,968</b>	<b>73,803</b>	<b>93,585</b>	<b>105,304</b>	<b>122,495</b>
Debt	1,768	1,824	4,245	4,245	4,245	4,245
Trade and other payables	54,465	72,528	66,611	86,086	97,549	114,364
Finance lease liabilities	3,465	3,616	2,947	3,254	3,510	3,885
<b>Longterm liabilities, total</b>	<b>161,461</b>	<b>166,105</b>	<b>105,122</b>	<b>107,933</b>	<b>110,274</b>	<b>113,707</b>
Debt	131,618	136,598	78,154	78,154	78,154	78,154
Finance lease liabilities	29,843	29,507	26,968	29,779	32,120	35,553
<b>Shareholders' equity</b>	<b>204,638</b>	<b>218,781</b>	<b>221,862</b>	<b>210,750</b>	<b>170,232</b>	<b>191,085</b>
<b>Total consolidated equity and debt</b>	<b>425,797</b>	<b>462,854</b>	<b>400,787</b>	<b>412,268</b>	<b>385,810</b>	<b>427,287</b>
<b>Ratios</b>						
Current ratio (x)	4.65	4.06	3.78	3.10	2.53	2.51
Quick ratio (x)	3.94	3.33	3.02	2.38	1.81	1.79
Net gearing	-20.5%	-19.8%	-25.7%	-31.0%	-14.2%	-21.4%
Book value per share (€)	0.29	0.30	0.30	0.29	0.23	0.26
Net debt	-41,960	-43,407	-57,084	-65,335	-24,114	-40,871
Return on equity (ROE)	6.9%	-5.0%	-5.0%	-5.1%	-21.3%	11.5%



## CASH FLOW STATEMENT

All figures in USD '000	2022A	2023A	2024A	2025E	2026E	2027E
<b>Profit before tax</b>	<b>14,987</b>	<b>-12,012</b>	<b>-8,513</b>	<b>-11,112</b>	<b>-40,518</b>	<b>28,104</b>
Depreciation, amortization, impairment	13,188	15,925	16,070	9,750	11,048	12,953
Gain on disposal of associate	-12,242	0	0	0	0	0
Equity-settled share-based payments	6,392	9,251	11,248	0	0	0
Fair value gain (loss) on revaluation	1,185	930	-4,990	0	0	0
Gain on disposal from PRV sale	0	-21,279	0	0	0	0
Other finance income	-4,485	-3,663	-6,820	0	0	0
Other finance expenses	5,463	9,069	9,887	0	0	0
Share of net profits in associates	1,083	289	1,758	0	0	0
Other	-1,576	-1,079	22	0	0	0
Changes in working capital	-387	-16,961	-10,072	19,454	-3,306	-4,850
Interest received, taxes paid	-1,150	2,228	-10,383	0	0	-7,251
<b>Operating cash flow</b>	<b>22,458</b>	<b>-17,302</b>	<b>-1,793</b>	<b>18,092</b>	<b>-32,776</b>	<b>28,956</b>
Investment in tangible/intangible assets	-1,977	-1,464	1,302	-6,723	-5,849	-8,390
<b>Free cash flow</b>	<b>20,481</b>	<b>-18,766</b>	<b>-491</b>	<b>11,369</b>	<b>-38,625</b>	<b>20,566</b>
Proceeds from sale of associates	7,300	0	0	0	0	0
Proceeds on PRV sale	0	21,279	0	0	0	0
Purchases of marketable securities	0	-382,014	-284,314	0	0	0
Proceeds from sale of marketable securities	0	232,811	314,630	0	0	0
<b>Investing cashflow</b>	<b>5,323</b>	<b>-129,388</b>	<b>31,618</b>	<b>-6,723</b>	<b>-5,849</b>	<b>-8,390</b>
Debt financing, net	0	0	-30,385	0	0	0
Share-based compensation	2,281	8,133	5,579	0	0	0
Payment on contingent consideration	0	0	0	0	0	0
Payment of lease liabilities	-3,311	-5,126	-5,149	0	0	0
Interest on loans	-3,952	-4,046	-4,457	0	0	0
<b>Financing cash flow</b>	<b>-4,982</b>	<b>-1,039</b>	<b>-34,412</b>	<b>0</b>	<b>0</b>	<b>0</b>
<b>Net cash flows</b>	<b>22,799</b>	<b>-147,729</b>	<b>-4,587</b>	<b>11,369</b>	<b>-38,625</b>	<b>20,566</b>
Exchange rate effects, other	-7,381	2,128	-2,210	0	0	0
<b>Cash, start of the year</b>	<b>191,924</b>	<b>207,342</b>	<b>61,741</b>	<b>54,944</b>	<b>66,313</b>	<b>27,688</b>
<b>Cash, end of the year</b>	<b>207,342</b>	<b>61,741</b>	<b>54,944</b>	<b>66,313</b>	<b>27,688</b>	<b>48,254</b>
<b>EBITDA/share</b>	<b>0.04</b>	<b>0.00</b>	<b>0.00</b>	<b>0.00</b>	<b>-0.04</b>	<b>0.06</b>
<b>Y-Y Growth</b>						
Operating cash flow	-40.7%	n.m.	n.m.	n.m.	n.m.	n.m.
Free cash flow	-13.4%	n.m.	n.m.	n.m.	n.m.	n.m.
EBITDA/share	2.0%	-90.1%	n.m.	n.m.	n.m.	n.m.

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**PRICE TARGET DATES**

Unless otherwise indicated, current prices refer to the closing prices of the previous trading day.

**AGREEMENT WITH THE ANALYSED COMPANY AND MAINTENANCE OF OBJECTIVITY**

The present financial analysis is based on the author's own knowledge and research. The author prepared this study without any direct or indirect influence exerted on the part of the analysed company. Parts of the financial analysis were possibly provided to the analysed company prior to publication in order to avoid inaccuracies in the representation of facts. However, no substantial changes were made at the request of the analysed company following any such provision.

**ASSET VALUATION SYSTEM**

First Berlin's system for asset valuation is divided into an asset recommendation and a risk assessment.

**ASSET RECOMMENDATION**

The recommendations determined in accordance with the share price trend anticipated by First Berlin in the respectively indicated investment period are as follows:

Category		1	2
Current market capitalisation (in €)		0 - 2 billion	> 2 billion
Strong Buy <sup>1</sup>	An expected favourable price trend of:	> 50%	> 30%
Buy	An expected favourable price trend of:	> 25%	> 15%
Add	An expected favourable price trend of:	0% to 25%	0% to 15%
Reduce	An expected negative price trend of:	0% to -15%	0% to -10%
Sell	An expected negative price trend of:	< -15%	< -10%

<sup>1</sup> The expected price trend is in combination with sizable confidence in the quality and forecast security of management.

Our recommendation system places each company into one of two market capitalisation categories. Category 1 companies have a market capitalisation of €0 – €2 billion, and Category 2 companies have a market capitalisation of > €2 billion. The expected return thresholds underlying our recommendation system are lower for Category 2 companies than for Category 1 companies. This reflects the generally lower level of risk associated with higher market capitalisation companies.

**RISK ASSESSMENT**

The First Berlin categories for risk assessment are low, average, high and speculative. They are determined by ten factors: Corporate governance, quality of earnings, management strength, balance sheet and financial risk, competitive position, standard of financial disclosure, regulatory and political uncertainty, strength of brandname, market capitalisation and free float. These risk factors are incorporated into the First Berlin valuation models and are thus included in the target prices. First Berlin customers may request the models.

**RECOMMENDATION & PRICE TARGET HISTORY**

Report No.:	Date of publication	Previous day closing price	Recommendation	Price target
Initial Report	10 November 2009	€0.52	Buy	€0.70
2...48	↓	↓	↓	↓
49	9 March 2020	€1.11	Buy	€2.00
50	23 April 2020	€1.34	Buy	€2.00
51	19 May 2020	€1.34	Buy	€2.00
52	4 August 2020	€1.01	Buy	€1.80
53	18 July 2023	€1.12	Buy	€1.50
54	9 August 2023	€1.13	Buy	€1.50
55	28 August 2024	€0.73	Buy	€1.60
56	29 October 2024	€0.78	Buy	€1.70
57	Today	€0.73	Buy	€2.30

**INVESTMENT HORIZON**

Unless otherwise stated in the financial analysis, the ratings refer to an investment period of twelve months.

### UPDATES

At the time of publication of this financial analysis it is not certain whether, when and on what occasion an update will be provided. In general First Berlin strives to review the financial analysis for its topicality and, if required, to update it in a very timely manner in connection with the reporting obligations of the analysed company or on the occasion of ad hoc notifications.

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#### Legally required information regarding

- key sources of information in the preparation of this research report
- valuation methods and principles
- sensitivity of valuation parameters

can be accessed through the following internet link: <https://firstberlin.com/disclaimer-english-link/>

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