



Update Report

Pharming Group

Broader and Deeper



Pharming

Chief Research Analyst

Marcel Wijma MSc

+1 (917) 460 6185 (US)

+31 (6) 1848 4204 (NL)

m.wijma@leeuwenhoeck.com

<http://www.leeuwenhoeck.com>



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Name:	Pharming Group
Country:	The Netherlands
Price:	EUR 0.95
ISIN Code:	NL0010391025
Reuters Code:	PHARM.AS
Market Cap (EUR m):	624.2
EV (EUR m):	632.4
Cash & cash eq. (EUR m):	66.9
Shares outstanding (m):	657.0
Volume:	18.4 million
Free float:	98%
52-week Range:	0.62-1.62

(EUR m)	2016A	2017A	2018E
Total Revenues	15.9	89.6	126.6
Net (Loss)/Profit	(17.5)	(80.0)	49.3
Net loss per share (cents)	(4.2)	(16.0)	7.5
R&D costs	15.4	18.7	19.0
Cash increase/(decrease)	(0.2)	28.9	20.0
Cash and marketable sec.	32.1	60.0	75.0



Executive Summary

- Pharming Group is a Dutch based biopharmaceutical company and one of the first publicly traded biotech companies in Europe. The company is focused on the development of recombinant proteins for therapeutic use. Pharming's main platform is the development of human recombinant proteins through the generation of transgenic animals which express the human protein in their milk.
- Earlier this year, the company discussed the strategy for the coming years for the broadening of its pipeline both for RUCONEST® (rhC1INH) and new protein replacement products. In the next months, we expect that the company will initiate new clinical programs to expand the use of RUCONEST® in new indications (like contrast induced nephropathy) as well as start clinical trials new programs like alpha-glucosidase for Pompe Disease. Each of these programs address markets that dwarf the HAE market size.
- Last month, the company surprisingly received a complete response letter from the FDA in which the FDA requested an additional clinical trial to further evaluate the effectiveness of RUCONEST® in prophylaxis in patients with HAE. This does not mean that the company is not allowed to sell RUCONEST® for prophylactic use anymore. Specialists already are prescribing Ruconest for off label use in prophylaxis, especially following manufacturing issue with the blood plasma derived products of Shire and CSL Behring. Focus will lie on the development of better and more convenient administration options of RUCONEST®. We believe that is the key growth driver for the HAE market in the coming years.



- In 2018H1 revenues of Ruconest continued to increase and almost doubled compared to 2017H1. The company also generated a net profit of EUR 6.4 million compared to a loss of EUR 30.2 million in 2017H1. A further increase of both revenues and profit in 2018H2 is expected. The company currently has enough cash to finance its programs with both Ruconest and also the new programs in Pompe and Fabry. We expect the cash position to improve in 2018H2 towards EUR 80 million.
- We have increased our valuation for Pharming based on a further increase of profits and revenues from Ruconest, from EUR 1.6 billion to EUR 2.0 billion. We have now also put a value on some other programs like contrast induced nephropathy (CIN) and Pompe's Disease. This translates based on the fully diluted number of 657 million shares into EUR 3.11 per share.



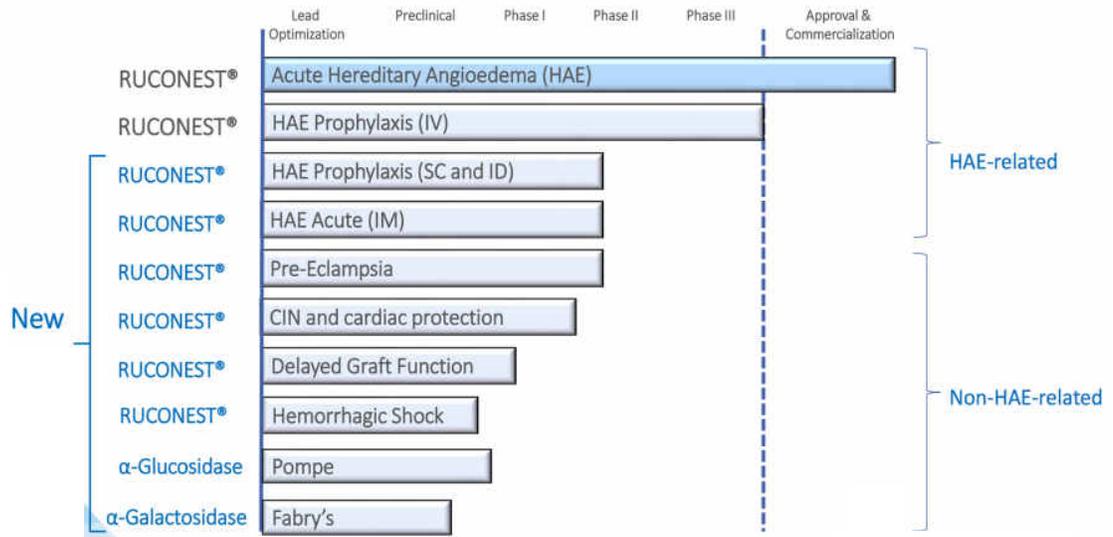
Pipeline: New Programs and New Indications

Pharming currently has a product portfolio which focuses on the commercialisation and further development of RUCONEST® (recombinant human C1-esterase inhibitor) for HAE, a genetic disorder. The Company is also evaluating RUCONEST® in other potential indications like pre-eclampsia and Contrast-induced Nephropathy (CIN) to generate value both in the short-term and long-term. Furthermore, Pharming has other recombinant protein assets (e.g. α -glucosidase and α -galactosidase) but these have not yet entered formal clinical trials. This summer, Pharming organized its first Capital Markets Day during which management discussed its ongoing activities and the strategy for its growing research and development pipeline both for its recombinant human C1 esterase inhibitor (rhC1INH) and new protein replacement products.

2018 will be driven by continued growth of RUCONEST®. It was also highlighted that the company sees three areas of growth for its pipeline:

- Improving RUCONEST® for the treatment of HAE, particularly by developing better and more convenient administration options for both acute treatment and prophylaxis.
- Developing RUCONEST® for other unmet medical needs like pre-eclampsia, Contrast-induced Nephropathy and Delayed Graft Function
- Developing new therapies for unmet medical needs other than HAE, like Pompe Disease and Fabry Disease.

As we already discussed HAE and its clinical data of RUCONEST® quite extensively in previous reports (see reports of August 2017 and May 2018), we will focus on the recent news flow about RUCONEST®, the development at competitors and in introduction into clinical programs for RUCONEST® (CIN) and other therapies (Pompe Disease). We expect short term added value from these two programs in the coming 6-12 months.

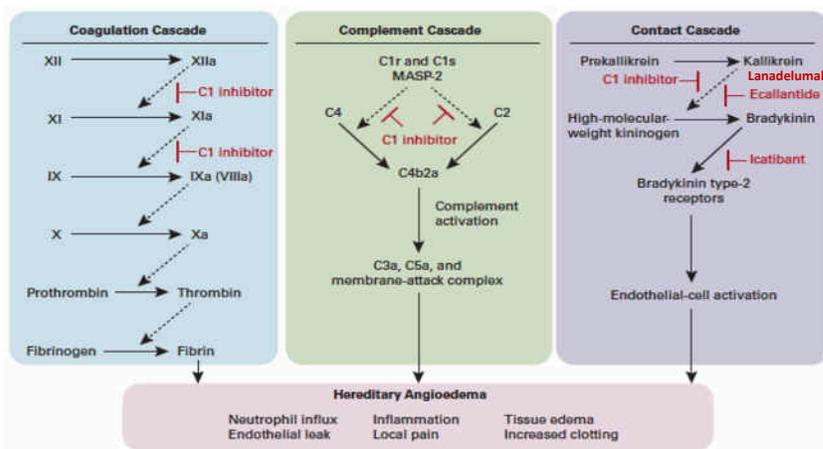


RUCONEST® (approved in Acute HAE, clinical development Prophylaxis HAE)

End of 2016, Pharming and its former US partner Valeant reached agreement for Pharming to acquire all North American commercialization rights to RUCONEST®, including all rights in the USA, Mexico and Canada. As a result of the acquisition of the rights for RUCONEST® in North America, Pharming was able to take the sales into its own hands and build up an own sales force. Currently, RUCONEST® is also sold for off-label use for prophylaxis in HAE patients. In November 2017, following feedback from FDA on two completed trials of RUCONEST® for prophylaxis of HAE attacks, Pharming filed an sBLA to expand the approved indication. The Phase 2 studies, an open-label study and a randomized, double-blind, placebo-controlled trial with 4-8 week treatment periods, showed consistent efficacy and safety results. In January 2018, FDA deemed the application as sufficiently complete to permit a substantive review of the Phase II data. However, last month the company received a complete response letter from the FDA in which it has requested an additional clinical trial to further evaluate the effectiveness of RUCONEST® in HAE prophylaxis. Although we did not expect this news, we feel that the impact on current and future



sales of RUCONEST® in prophylaxis use is rather limited. RUCONEST® is already widely prescribed off-label for prophylaxis use. There is also no question about the efficacy of RUCONEST® in both acute and prophylaxis use. We feel that the future growth of the HAE market is much more dependent on more convenient administration options for patients like subcutaneous administration versus intravenous administration. Pharming is currently working to develop a subcutaneous version of RUCONEST®, smaller vials and even a novel intradermal application via a newly developed patch. That would also differentiate RUCONEST® from its competitors, all of whom have painful injections. Even the newly approved antibody lanadelumab from Shire/Takeda. The latter product currently has the advantage that it is administered subcutaneously. However, we do not think this product to be superior to RUCONEST® when looking at effectiveness. On the contrary, lanadelumab is targeting plasma kallikrein and therefore inhibiting the activity of kallikrein. By doing so, this medication prevents the cleavage of high molecular weight kininogen and the release of bradykinin that leads to symptomatic HAE attacks. The C1 inhibitor RUCONEST® controls activation in the complement, coagulation, and contact cascades, and all three cascades are dysregulated in hereditary angioedema. Replacement of C1 inhibitor restores homeostasis. Lanadelumab, among others, specifically inhibit the contact cascade but have no direct effect on the complement or coagulation cascades.



Source: Pharming, Van Leeuwenhoek



If lanadelumab would be the new golden standard for treating HAE, then this would alter the preferred mechanism of action. The trials with lanadelumab showed that still a quarter of all the patients experience acute attacks. That would make an alternative drug still very necessary and then RUCONEST® would be that alternative. Therefore, also according to market research organization EvaluatePharma, RUCONEST® is expected to be the second best selling drug in HAE after lanadelumab (see graph below).

Product	Company	Pharma class	Admin.	Indication	2018E \$m	2020E \$m	2022E \$m	2024E \$m
Lanadelumab	Shire	Anti-plasma kallikrein MAb	Subcut.	Prophylactic	70	645	1161	1569
Ruconest	Pharming	C1 esterase inhibitor	Intraven.	Episodic	162	292	452	614
Haegarda	CSL	C1 esterase inhibitor	Subcut.	Prophylactic	207	311	375	319
Cinryze	Shire	C1 esterase inhibitor	Intraven.	Prophylactic	658	518	376	288
Beriner P	CSL	C1 esterase inhibitor	Intraven.	Episodic	350	241	176	227

Source: EvaluatePharma

Nonetheless, pricing of lanadelumab still remains an issue. The US pricing watchdog Icer has already begun evaluating the project, along with other prophylactic HAE therapies, with a report expected in October. Analysts say the Icer report and resistance from payers are among the biggest potential stumbling blocks for lanadelumab. Annual price would boil down to more than USD 500,000 for lanadelumab.

RUCONEST® in Contrast-induced Nephropathy (CIN)

Iodinated contrast media (CM) are an essential component of contemporary imaging and interventional studies. Although CM are generally well tolerated, they have been causally linked to



acute kidney injury known as contrast-induced nephropathy (CIN). CIN was first described during the 1950s in case reports of fatal acute renal failure that had occurred following intravenous pyelography in patients with renal disease arising from multiple myeloma. Contrast-induced nephropathy (CIN) is widely recognised as the third most common cause of hospital acquired acute kidney injury (AKI) and accounts for 11%-12% of all cases of in-hospital AKI and an in-hospital mortality rate of 6%. CIN occurs after intravascular administration of iodinated contrast media during diagnostic and/or interventional procedures. The risk of development of CIN is highest with coronary angiography and percutaneous coronary intervention (PCI). CIN occurs in about 14.5% of patients after coronary interventions with inhospital mortality rate of 7.1% in patients without the need for dialysis and 35.7% in those requiring dialysis. CIN is uncommon in patients with normal baseline renal function. It occurs more frequently in patients with preexisting renal impairment particularly if it is associated with diabetes. CIN is defined as an acute deterioration of renal function after intravascular exposure to contrast media in absence of other causes. The serum creatinine levels begin to rise within 24-48 hours, peak at 2-3 days and return to the baseline values within 2 weeks. The most commonly used definition of CIN in the literature is either a relative increase in serum creatinine of 25% or an absolute increase of 0.5 mg/dL from a baseline value within 48 to 72 hours after contrast exposure. Additionally, there must be no other alternative cause for the elevation of serum creatinine levels and it must persist for 2-5 days. Apart from intravenous hydration preventive strategies for CIN are lacking.

The complement system consists of several circulating proteins that are implicated in the first-line defence against pathogens and in the removal of dying cells. Following renal ischemia activation of the lectin pathway of complement in particular has been associated with local tissue damage in the kidney. RUCONEST® markedly reduced tissue damage in experimental models of renal ischemia and reperfusion injury, but has not been investigated in human ischemia.

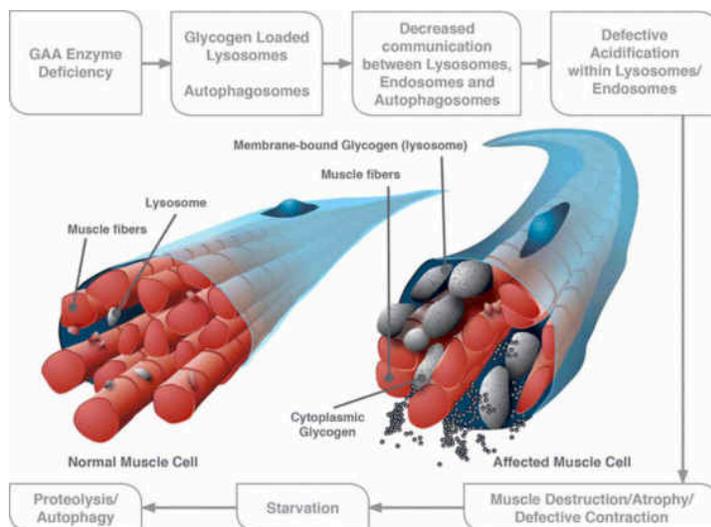


In January 2017, Pharming started a investigational Phase II trial with 80 patients in Basel Switzerland, also called the PROTECT study (Prevention of Contrast-induced Nephropathy in High-risk Subjects). The study is a randomized, placebo-controlled, double-blind single-center trial that assessed the effect of prophylactic administration of RUCONEST® on the degree of acute kidney injury subjects undergoing elective coronary angiography. Patients with an estimated glomerular filtration rate ≤ 50 ml/min/1.73 m² and at least one additional risk factor for CIN were enrolled and randomly assigned to 1) Conestat alfa at 50 U/kg given as intravenous injection immediately before and 4 hours after coronary angiography or 2) placebo (sodium chloride). All patients will receive standard intravenous hydration with isotonic saline. Surrogate markers of kidney injury including serum creatinine and cystatin C and urinary Neutrophil gelatinase-associated lipocalin and TIMP2 * Insulin-like growth factor-binding protein 7 (IGFBP7), were assessed over a 48 hours time period. In addition, increases in troponin T, a marker of cardiac damage, will be assessed. Patients are followed for thromboembolic, anaphylactic and a composite endpoint of cardiovascular and renal events over a 12 week period. The primary outcome measure is peak change in urinary Neutrophil gelatinase-associated lipocalin within 48 hours after elective coronary angiography. The study was completed in July and we expect data this month. As a follow up, Pharming indicated that it will initiate a formal Phase II study in CIN if the primary marker gives a positive signal. This would provide a clear therapeutic window to offset the risk of renal damage in patients that run this risk.

Alpha-glucosidase in Pompe disease (PGN004)

Pompe disease is a rare inherited neuromuscular disorder that causes progressive muscle weakness in people of all ages. The disease is named after Johannes C. Pompe, a Dutch doctor who first described the disorder in 1932 in an infant patient. However, Pompe can affect people of all ages, with symptoms first occurring at any time from infancy to adulthood.

Pompe disease is caused by a defective gene that results in a deficiency of an enzyme, acid alpha-glucosidase (GAA). This enzyme is required to breakdown (metabolise) the complex carbohydrate glycogen and convert it into the simple sugar glucose. Glycogen is a thick, sticky substance and failure to properly break it down results in massive accumulation of lysosomal glycogen in cells, particularly in cardiac, smooth, and skeletal muscle cells.



Pathophysiology of late-onset Pompe disease. Abbreviations: GAA, acid alpha-glucosidase

Additional abnormalities may include enlargement of the heart (cardiomegaly), the liver (hepatomegaly), and/or the tongue (macroglossia). Without treatment, progressive cardiac failure usually causes life-threatening complications by the age of 12 to 18 months. Pompe disease can also present in childhood, adolescence or adulthood, collectively known as late-onset Pompe disease. The disease is estimated to affect 1 in every 40,000 individuals. The only approved therapy to date is Enzyme Replacement Therapy (ERT) wherein recombinant human α -glucosidase, produced on Chinese Hamster Ovary (CHO) cells (Myozyme®/Lumizyme® from Genzyme (acquired by Sanofi), is administered intravenously (IV) every 2 weeks with a dosing of 20 mg/kg body weight. Patients receiving ERT need treatment during their entire life. The major drawbacks



in ERT are immune responses which can be raised towards an impure recombinant protein and low efficacy due to limited ability of the protein to reach and bind to its specific receptors on the into target cells, which seems to be the main reason for the high dosing. Several alternatives to Myozyme are under development, including a yeast derived α -glucosidase with an improved glycosylation pattern for better recognition by cellular receptors (Oxyrane) and a gene therapy approach by Duke University.

Human recombinant α -glucosidase has been produced in transgenic animals before. Until 2002, Genzyme together with Pharming generated transgenic rabbits producing α -glucosidase. Production levels at the time were as high as 8 g/L (Bijvoet et al. 1998, 1999). The transgenic material was shown to be active in clinical trials. In 2002 all assets related to the α -glucosidase program were transferred to Genzyme under the Settlement Arrangements of 15 August 2002. Genzyme then stopped the program, preferring to continue with the better-understood CHO-cell program which GAA became Myozyme®, but scaling issues forced it to develop a second cell-line version to achieve capacity, which became Lumizyme®. Both products carry a boxed warning for immunogenicity. Given insights and experience gained with RUCONEST®; a similarly highly glycosylated protein, Pharming is aiming to develop a less immunogenic GAA from its transgenic rabbit platform. than Myozyme®/Lumizyme®. The product will not be considered a 'Biosimilar' by the authorities as it is produced on a totally different production platform, but from an activity and safety perspective, this new product will be broadly biosimilar to Myozyme®/Lumizyme®. The approach by Pharming (if successful) may also result in a so-called 'Biobetter'. In 2017, sales of Myozyme®/Lumizyme® were EUR 789 million, an increase of 10.1%. On this basis, assuming a similar growth for the products in 2018, the size of the Pompe disease market globally may be estimated at approximately EUR 1-1.3 billion. Recently, Pharming indicated that it plans to initiate a Phase I/II trial in Pompe Disease in the beginning of 2019. We believe that, considering the existing safety data, this trial can be concluded within 12 months, followed by a Phase II/III trial in 2020H2.



Financials

For 2018H1 ended 30 June 2018, net product sales increased 96% to EUR 59.1 million compared to EUR 30.1 million in the same period last year. The positive sales momentum in the USA continued in 2018Q2, following higher than expected sales in 2018Q1 as a result of the shortage of Cinryze from Shire, with net sales of USD 33.9 million in Q2 (USD 34.3 million in Q1) despite stock level adjustments and a weakening in the exchange rate between US dollars and euros. As the clearest measure of the success of RUCONEST®, the number of patients using the product regularly in the USA has been increasing steadily since the company reacquired the commercial rights in 2016. Operating income improved 288% to a profit of EUR 16.3 million from a loss of EUR 4.2 million in 2017H1. The company has invested in expanding the pipeline for RUCONEST® and for its follow-up programs in Pompe disease and Fabry's disease, the costs of which are reflected in a flat operating profit for 2018Q2.

For the whole year we expect an ongoing strong growth in revenues from sales of RUCONEST® due to ongoing investments in sales & marketing in the US. For 2018FY we estimate that Pharming will generate sales of EUR 126.5 million and further increasing in 2019 to EUR 182 million and in 2020 to EUR 225 million. On the operating level, the company already reached profitability last year, whereas in 2018H1, it also managed to become profitable bottom line. The company managed to change a net loss of EUR 30.2 million in 2017H1 to a net profit of EUR 6.4 million in 2018H1. The improvement was related to strong growth in sales over the last 12 months and the elimination of the financial expenses associated with the refinance in 2017.

The total cash and cash equivalent position (including restricted cash) increased by EUR 6.9 million from EUR 60.0 million at 2018Q1 to EUR 66.9 million at the end of 2018H1. The increase in cash is consistent with the underlying growth in product sales. From 2018Q3 onwards, Pharming will be making quarterly repayments of its outstanding debt facility to Orbimed and so we expect cash to



decrease slowly over the rest of the year.

Profit & Loss Statement

EUR million	2016A	2017A	2018H1A	2018E	2019E	2020E
Total Revenues	15.9	89.6	59.5	125.6	159.7	213.2
Cost of Sales	4.7	12.4	9.5	16.7	19.2	25.6
Gross Profit	11.2	77.2	50.0	108.9	140.5	187.6
R&D Costs	15.4	18.7	12.0	22.0	24.0	25.0
G&A Costs	4.6	6.0	5.2	9.0	10.0	12.0
Marketing & Sales	3.0	31.4	16.7	45.8	71.4	38.4
Operating Profit	(11.5)	21.9	16.3	28.4	56.1	112.2
Financial Income/(Expenses)	(6.0)	(111.3)	(9.0)	(16.0)	(20.0)	(25.0)
Net Profit/(Loss)	(17.5)	(89.4)	6.4	12.4	36.1	87.2

Consolidated statement of cash flows

EUR million	Dec 31 st 2017A (12 months)	Dec 31 st 2018E (12 months)
Cash flow from operating activities	38.2	20.0
Cash flow from investing activities	(6.0)	(5.0)
Cash flow from financing activities	(3.3)	(3.0)
Cash and cash equivalents at beginning of the period	32.1	60.0
Net change in cash and cash equivalents	28.9	15.0



Valuation

Based on our NPV based valuation, we believe that Pharming is substantially undervalued at the current share price of EUR 0.95. We have increased our valuation for the company, considering the fact that we have valued new programs at Pharming, being the development of RUCONEST® for Contrast-induced Nephropathy (CIN) the company's current total value should increase from EUR 1.6 billion to EUR 2.0 billion, which translates, based on an expected number of outstanding shares of approximately 657 million, into EUR 3.11 per share. At this moment we do not address value to other programs in Pharming's pipeline. This conservative approach offers potential upside for the share price.

Valuation Revised Upwards: From EUR 2.40 per share to EUR 3.11

Based on our NPV based valuation, we believe that Pharming is substantially undervalued at the current share price of EUR 0.96. We have increased our valuation for the company taking into account new programs for RUCONEST® and other indications like Pompe's Disease. Other programs like pre-eclampsia we have not yet considered due to a lack of information that we can use to make a credible valuation on these programs. The company's current total value should increase from EUR 1.6 billion million to EUR 2.0 billion, which translates, based on an expected number of outstanding shares of approximately 657 million, into EUR 3.11 per share.

Valuation RUCONEST in Acute and Prophylactic HAE

In estimating a value for RUCONEST®, we considered potential markets in the US and Europe with the US market calculated to be 75-85% of the total market. We calculate a Risk adjusted Discount Rate of 9%. Pricing per attack is set at USD 10,000 with an average of 25 attacks per year. We calculate a net margin rising to 60-70% within a few years. We estimate that a peak



market share of 20-25% for acute HAE and 15% for prophylactic HAE should be possible.

Year	2018	2019	2020	2021	2022	2023	2024	2025	2026	2027	2028
Market Size US Acute HAE	1069	1112	1168	1226	1287	1352	1419	1490	1565	1643	1725
Penetration	10,0%	11,5%	13,0%	15,0%	16,5%	19,0%	20,0%	19,0%	18,0%	17,0%	16,0%
Market Size US Prophylactic	875	901	928	956	985	1014	1045	1076	1109	1142	1176
Penetration	1,0%	1,5%	3,0%	4,0%	5,5%	6,0%	6,5%	7,5%	8,0%	9,0%	10,0%
Total Revenue US&EU (EURm)	€ 125,4	€ 155,8	€ 207,7	€ 258,5	€ 315,0	€ 368,7	€ 404,8	€ 422,3	€ 429,5	€ 444,8	€ 458,6
Margin up to 65%	34,1	51,4	87,3	133,4	196,5	232,4	259,7	277,0	288,3	303,6	315,6
WACC 9%	1,00	0,92	0,84	0,77	0,71	0,65	0,60	0,55	0,50	0,46	0,42
NPV (million)	34,1	28,8	73,4	87,6	121,5	151,1	154,9	151,5	144,7	139,8	133,3
Total NPV (million)											1,679
Value per share (EUR)											2.56

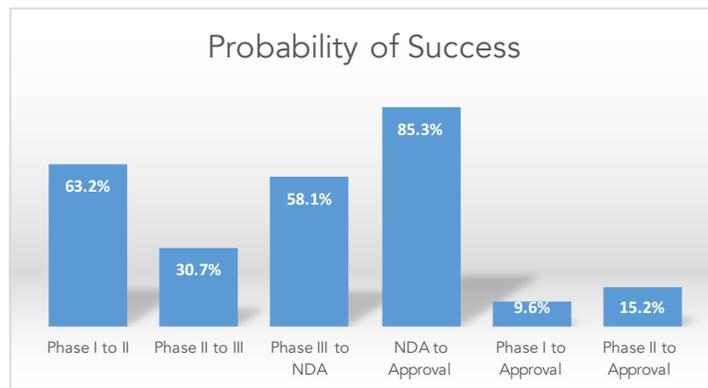
Phase Success and Likelihood of Approval (LOA)

In estimating a value for the new clinical programs with RUCONEST® in CIN and the Pompe program, we made use of several studies that were done on the clinical development success rates for investigational drugs to measure success rates for investigational drugs. We analyzed individual drug program phase transitions from January 1, 2006 to December 31, 2015. For the ten years studied, 9,985 transitions in the Biomedtracker database were analyzed. A phase transition is the movement out of a clinical phase – for example, advancing from Phase I to Phase II development, or being suspended after completion of Phase I development. These transitions occurred in 7,455 clinical drug development programs, across 1,103 companies (both large and small), making this the largest study of its kind. With this broad set of data, we aimed to capture the diversity in drug development across levels of novelty, molecular modalities, and disease indications. Only company-sponsored, FDA registration-enabling development programs were considered; investigator-sponsored studies were excluded from this analysis.

The Phase I transition success rate was 63.2% (n=3,582). As this Phase is typically conducted for safety testing and is not dependent on efficacy results for candidates to advance, it is common for



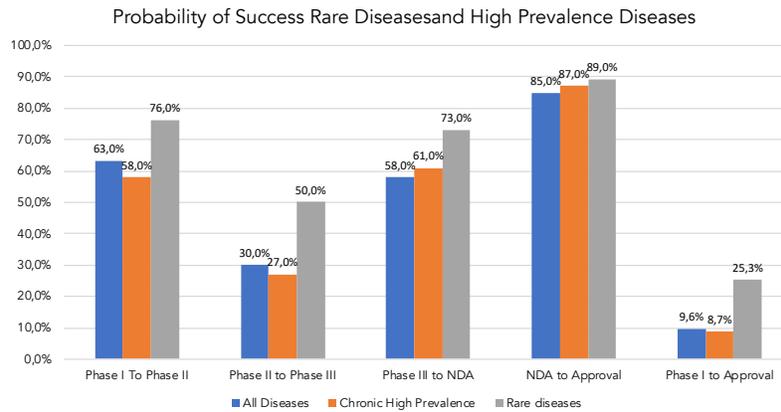
this phase to have the highest success rate among the clinical phases across most categories analyzed in this report. Phase I success rates may also benefit from delayed reporting bias, as some larger companies may not deem failed Phase I programs as material and thereby not report them in the public domain. The Phase II transition success rate (30.7%, n=3,862) was substantially lower than Phase I, and the lowest of the four phases studied. As this is generally the first stage where proof-of-concept is deliberately tested in human subjects, Phase II consistently had the lowest success rate of all phases. This is also the point in development where industry must decide whether to pursue the large, expensive Phase III studies and may decide to terminate development for multiple reasons including commercial viability. The second-lowest phase transition success rate was found in Phase III (58.1%, n=1,491). This is significant as most company-sponsored Phase III trials are the longest and most expensive trials to conduct. The probability of FDA approval after submitting a New Drug Application (NDA) or Biologic License Application (BLA), taking into account re-submissions, was 85.3% (n=1,050). Multiplying these individual phase components to obtain the compound probability of progressing from Phase I to U.S. FDA approval (LOA) reveals that only 9.6% (n=9,985) of drug development programs successfully make it to market (see graph below)



Source: BIO Industry Analysis



In recent years, there has been an increase in funding for companies focused on rare diseases. This is welcome news as there are reportedly 7,000 rare diseases and most do not have an approved therapeutic treatment. One question that is often asked is if the probabilities of success are any better for rare diseases, especially for those in which a particular defective gene has been confirmed as the sole contributor. With programs from both groups identified, we compared phase transition success rates and LOA as shown in the graph below. At 25.3%, the overall LOA from Phase I for Non-Oncology rare diseases was 2.6x higher than the LOA for all diseases and 3x higher than the 8.7% LOA for chronic, high prevalence diseases.



Source: BIO Industry Analysis

Valuation RUCONEST in Contrast-induced Nephropathy (CIN)

For the valuation of RUCONEST® in CIN we made several assumptions. Assuming that positive data from the Basel investigational Phase II trial and the start of a new Phase II in 2019H1, we believe that market introduction is possible in 2021. Based on several reports from the US National Center for Health and the EU, we went with 32.5 million hospitalizations in the US per year or 10% of the total population. For the EU, we calculated 20 million hospitalizations per year. Around 2.2-2.5% of all hospitalizations lead to Hospital Acquired Acute Kidney Injury (HAAKI). Based on research it was determined that roughly 12% of all HAAKI events was a result of CIN. We priced



the product at USD 12,000 per treatment in the US and USD 8,000 in the EU. Based on the report from BIO, we worked with a LOA of 50%. The discount rate was calculated at 13%. This leads to a total current value of the RUCONEST® program in CIN of EUR 190 million or EUR 0.29 per share.

Year	2021	2022	2023	2024	2025	2026	2027	2028	2029	2030	2031
Market Size CIN	1540	1547	1555	1563	1571	1579	1586	1594	1602	1610	1618
Penetration	1%	2%	4%	8%	12%	16%	20%	22%	24%	25%	24%
Total Revenue US&EU	7,7	30,9	62,2	125,0	188,5	252,6	317,3	350,8	384,6	402,6	388,4
Margin up to 65%	3,8	15,5	31,1	62,5	94,2	126,3	158,6	175,4	192,3	201,3	194,2
WACC 13%	0,7	0,6	0,6	0,5	0,4	0,4	0,3	0,3	0,3	0,2	0,2
NPV (million)	2,7	9,7	17,3	30,8	41,3	49,2	55,0	54,0	52,6	49,0	42,0
Total NPV (million) EUR											190,5
Value per share (EUR)											0.29

Valuation Alpha-glucosidase in Pompe's disease

For the valuation of alpha-glucosidase in Pompe's Disease we also made several assumptions. Assuming that Pharming will start a new Phase I/II in 2019H1, we believe that market introduction is possible in 2022. The prevalence of Pompe's Disease is roughly 1 in 40,000 persons. We calculate an addressable market of 40% with a peak market share of 25%, which is rather conservative. We priced the product at USD 300,000 per treatment in the US and USD 240,000 in the EU. Based on the report from BIO, we worked with a LOA of 25%. The discount rate was calculated at 13%. This leads to a total current value of the alpha-glucosidase program in Pompe's Disease of EUR 174 million or EUR 0.26 per share.

Year	2022	2023	2024	2025	2026	2027	2028	2029	2030	2031	2032
Market Size Pompe	3433	3484	3536	3589	3643	3698	3753	3810	3867	3925	3984
Addressable Market 40%	1480	1554	1632	1713	1799	1889	1984	2083	2187	2296	2411
Penetration	2,0%	5,0%	8,0%	12,0%	15,0%	18,0%	20,0%	22,0%	23,0%	24,0%	25,0%
Total Revenue US&EU	\$37,7	\$98,2	\$163,9	\$256,3	\$334,2	\$418,	\$485,1	\$556,9	\$607,7	\$662,	\$720,0
Margin up to 65%	22,6	58,9	98,3	153,8	200,5	251,0	291,1	334,1	364,6	397,2	432,0
WACC 13%	0,64	0,57	0,51	0,45	0,40	0,36	0,32	0,29	0,26	0,23	0,20
NPV (million)	14,4	33,4	49,8	69,6	81,0	90,5	93,7	96,1	93,6	91,0	88,4
Total NPV (million) EUR											174,2
Value per share (EUR)											0.26



Analyst: Marcel Wijma MSc

Marcel Wijma, Chief Research Officer and managing partner, has a longstanding history in financial biotech research. After selling Van Leeuwenhoek Research (VLR) to SNS Securities in 2006, he established an award winning analyst team in biotech/life sciences at SNS Securities. In 2009, Marcel was awarded by Financial Times/Starmine as being one of the Top-3 biotech analysts in Europe. Later that year, Marcel purchased VLR from SNS Securities after which the company was reconstituted. At VLR, he leads the professional VLR research organisation, which is augmented by selected external financial researchers with a specialisation in Life Sciences. Mr. Wijma has a Masters degree in Financial Economics from Erasmus University in Rotterdam.

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