Update Report

Pharming Group

Rerating Justified

Chief Research Analyst

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Date: 28 May 2020

Name:	**Pharming Group**
Country:	**The Netherlands**
Price:	**EUR 1.35**
ISIN Code:	**NL0010391025**
Reuters Code:	**PHARM.AS**
Market Cap (EUR m):	**857.3**
EV (EUR m):	**874.4**
Cash & cash eq. (EUR m):	**136.1**
Shares outstanding (m):	**635**
Volume:	**18.33 million**
Free float:	**98%**
52-week Range:	**0.71-1.64**

(EUR m)	2018A	2019A	2020E
Total Revenues	135.1	169.0	215.0
Net (Loss)/Profit	25.0	36.2	57.1
Net profit per share (cents)	3.8	5.4	9.0
R&D costs	28.9	28.4	37.9
Cash increase/(decrease)	18.6	(12.0)	113.0
Cash and marketable sec.	81.5	68.6	182.5

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.

- The company's lead product RUCONEST® for acute use in HAE continues to show strong growth despite initial fears that the introduction of Takeda's Takhzyro for prophylactic use in HAE would lower market share. On the contrary, we believe that it has led to a higher use of RUCONEST for acute use. Besides, Pharming has made substantial investments in the expansion of its production capacity and clinical development costs for the new indications for RUCONEST®.

- In the past year, the company has made important steps in the development of its pipeline. The company has initiated the first clinical study of RUCONEST® in pre-eclampsia and the start of a Phase II study of acute kidney injury is imminent. These studies extend the use of RUCONEST® in other indications that offer much larger potential markets compared to HAE. In the coming years, a further deepening of its technology platform, will be start of clinical trials in developing improved treatments for rare diseases like Pompe and Fabry.

- Next to further developing its proprietary technology platform for other indications, the company made an important step with acquiring an exclusive license from Novartis to leniolisib, a late stage drug for the treatment of AFDS, an ultra-rare debilitating disease with no approved treatment. If approved the drug is expected to be on the market in 2021H2. We

expect the drug to be a great fit for the company's existing commercial infrastructure.

- In 2020Q1, revenues of RUCONEST® came in at EUR 49.3 million, an increase of 40% yoy. Net profit amounted to EUR 8.4 million (+25%) with a cash position of EUR 136.1 million despite paying a EUR 18.1 million milestone to Bausch Health. We expect a further increase in both revenues and profit in 2020 is expected. Following a recent placement of EUR 125 million of senior unsecured convertible bonds due 2025, the company currently has enough cash to finance its programs with RUCONEST® and also the new programs in Pompe and Fabry. We expect the cash position in 2020 to be north of EUR 175 million.

- In April, the company announced encouraging results from five patients with confirmed COVID-19 infections hospitalized with related severe pneumonia that were treated with RUCONEST® at the University Hospital Basel, Switzerland. Following these results, a multinational, randomized, controlled, investor-initiated clinical trial with up to 150 patients with confirmed COVID-19 infections, is planned. The pandemic does not have any impact on the production, availability and distribution of RUCONEST®. On the other side, COVID-19 has resulted in halting recruitment of new patients in ongoing clinical trials. This leads to delays in the timelines for the pre-eclampsia and acute kidney injury studies.

- We have increased our valuation for Pharming from EUR 2.0 billion to EUR 2.1 billion. This is based on a further increase of profits and revenues from RUCONEST® as well as the ongoing development of its late stage pipeline including leniolisib. We have now also put a value on other programs in clinical development like pre-eclampsia and contrast induced nephropathy (CIN) Furthermore, we expect Pompe's Disease to be in the clinic in the near future. This translates based on the fully diluted number of 742 million shares into EUR 2.84 per share.

Pipeline: New Programs and New Indications

Pharming currently has a product portfolio which focuses on the commercialization and further development of RUCONEST® (recombinant human C1-esterase inhibitor) for HAE, a genetic disorder. The Company also started clinical trials with RUCONEST® in pre-eclampsia and will soon initiate clinical trials in acute kidney injury to generate value both in the short-term and long-term. With obtaining an exclusive license from Novartis on leniolisib in activated PI3K-delta syndrome (APDS), Pharming manages to broaden its pipeline that also offers a strategic fit to its existing medical and commercial infrastructure. Furthermore, Pharming has other recombinant protein assets (e.g. **α**-glucosidase and **α**-galactosidase) but these have not yet entered formal clinical trials. 2020 will be driven by continued growth of RUCONEST®. The Company sees four 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.

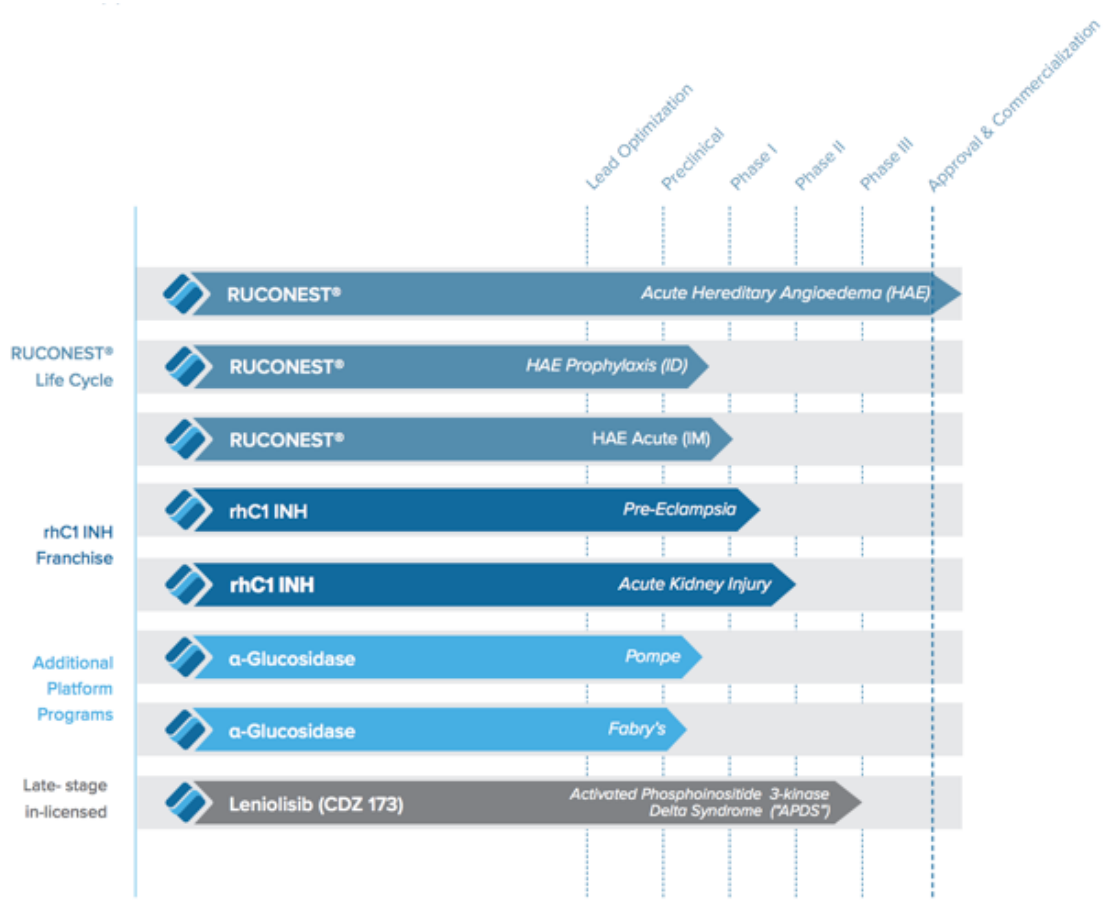
- Developing RUCONEST® for other unmet medical needs like pre-eclampsia, Acute Kidney Injury and Delayed Graft Function

- Developing new therapies for unmet medical needs other than HAE, like Pompe Disease and Fabry Disease.

- Additional acquisitions and/or in-licensing opportunities following the recently acquired rights on leniolisib from Novartis.

As we already discussed HAE and the clinical data of RUCONEST® quite extensively in previous reports, we will focus on the recent news flow about RUCONEST®, the development at competitors and in introduction into clinical programs for RUCONEST® (AKI and pre-Eclampsia)

and other therapies (Pompe Disease and APDS). Finally, we also will give our opinion about a potential acquisition(s).

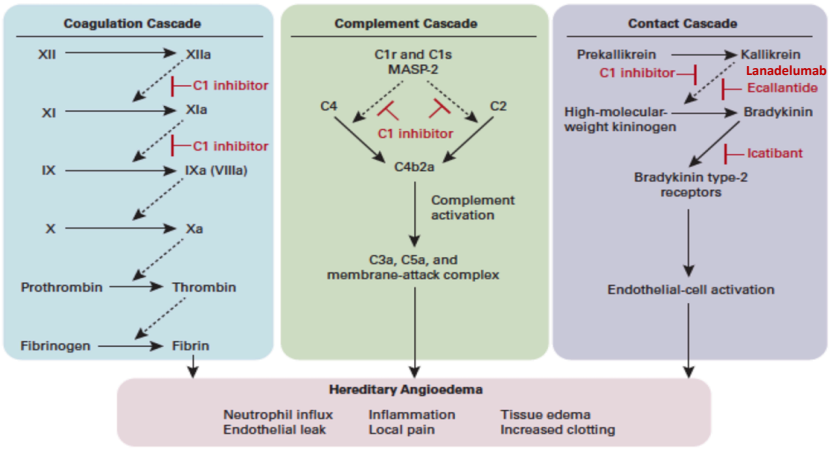


Source: Pharming

RUCONEST® in Acute HAE

End of 2016, Pharming and its former US partner Valeant reached an 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.

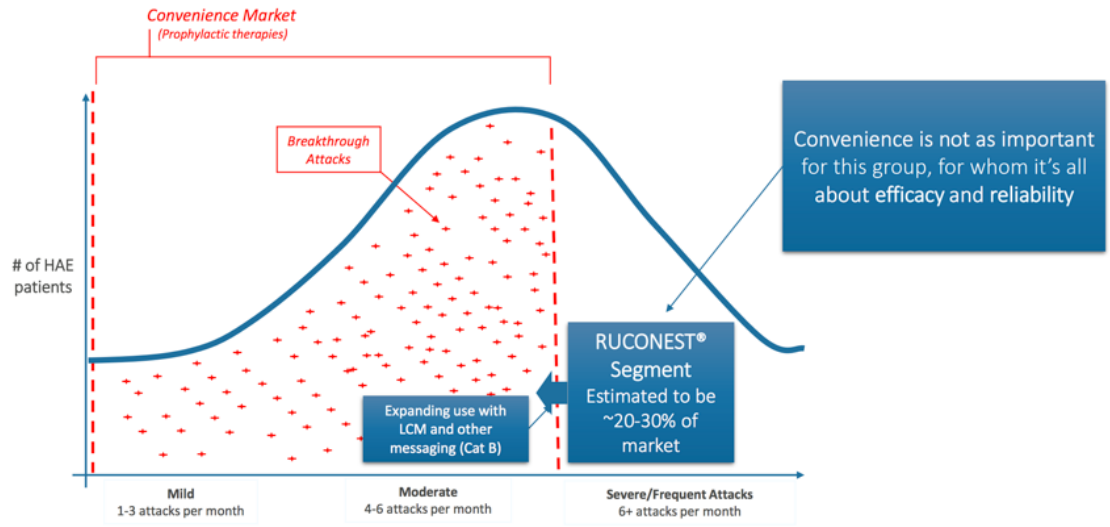
Despite the increasing competition in the US, particularly with the approval of Takhzyro from Shire/Takeda for prophylactic use in HAE, Pharming manages to maintain its position and even strengthen it. The latter product currently has the advantage that it is administered subcutaneously. However, we do not think this product to be superior to RUCONEST® when looking at effectiveness. On the contrary, Takhzyro 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. Takhzyro, among others, inhibits the contact cascade explicitly but has no direct effect on the complement or coagulation cascades.



Source: Pharming, Van Leeuwenhoeck

Experience with Takhzyro showed that still, a quarter of all the patients experience acute attacks. That makes an alternative drug necessary. Patients with HAE, therefore, also need to have vials of RUCONEST® and/or CSL's plasma product Berinert at their disposal at home. The increasing demand for Takhzyro for prophylactic use would also mean a growing demand for RUCONEST®.

For the same reason, we feel that BioCryst Pharmaceuticals (BCRX) oral drug berotralstat for prophylactic use (filed December 2019) will also not hurt RUCONEST® despite its more convenient use (oral drug). Patients with moderate to severe attacks mainly use RUCONEST®. Efficacy and reliability for this group of patients are of more importance than convenience.



Source: Banerji A, et al. Allergy Asthma Proc. 2018;39;212-223

RUCONEST® Pre-eclampsia

Pre-eclampsia is a condition during pregnancy where there is a sudden rise in blood pressure and swelling, mostly in the face, hands, and feet. Pre-eclampsia is the most common complication to occur during pregnancy. It generally develops during the third trimester and affects about 1 in 20 pregnancies. Although 6 to 8 percent of all pregnant women experience high blood pressure, it does not necessarily mean they have pre-eclampsia. The most telling sign is the presence of protein in the urine. As the pre-eclampsia progresses, the woman may experience fluid retention (edema), with swelling in the hands, feet, ankles, and face. Swelling is a common part of pregnancy, especially during the third trimester, and tends to occur in the lower parts of the body, such as the

ankles and feet. Symptoms are typically milder first thing in the morning and build up during the day. This is not pre-eclampsia, in which edema occurs suddenly and tends to be much more severe. When it arises, the condition begins after 20 weeks of pregnancy. In severe disease, there may be red blood cell breakdown, a low blood platelet count, impaired liver function, kidney dysfunction, swelling, shortness of breath due to fluid in the lungs, or visual disturbances. Pre-eclampsia increases the risk of poor outcomes for both the mother and the baby. If left untreated, it may result in seizures, at which point it is known as eclampsia.

In September 2018, Pharming filed a clinical trial application with the European Medicines Agency (EMA) to initiate the clinical development of RUCONEST® to treat and prevent pre-eclampsia (PE). As part of this process, the study was submitted to and has just been cleared by the first investigating center's ethics committee. A similar clinical trial application has been filed with the Therapeutic Goods Administration (TGA) in Australia, where it is still subject to ethics committee clearance. The first part of the study is an open-label trial to investigate the tolerability and safety of treatment with RUCONEST®. Pharming has begun recruiting a small number of patients in this part of the study in mid- to late-stage symptomatic pre-eclampsia, where women at 27 weeks' term or later may receive RUCONEST® from their first apparent symptoms of PE for the remainder of their pregnancy.

To date, RUCONEST® has been used to treat HAE in approximately 50 pregnant HAE patients (up to 40 times during a single pregnancy), including during delivery, with no safety issue for either mother or baby detected at the time or since.

RUCONEST® in Acute Kidney Injury (AKI)

Iodinated contrast media (CM) are an essential component of contemporary imaging and interventional studies. Although CM is generally well tolerated, it has 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. CIN is widely recognized as the third most common cause of hospital-acquired acute kidney injury (AKI). It 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 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 an in-hospital 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 the 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 defense against pathogens and 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 an 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. In October 2018, Pharming announced the positive results of this Phase II trial. In the overall study, RUCONEST® showed a statistically-significant effect (p= 0.038) in reducing the rise in urinary Neutrophil Gelatinase-Associated Lipocalin (NGAL), the primary endpoint for the study and a generally recognized early marker of acute renal injury, in patients with diagnosed renal function impairment undergoing interventions enhanced with standard contrast media such as PCIs. The results were especially evident in the sub-group of patients (n=30) undergoing PCI. The intent-to-treat analysis in this group showed that patients on RUCONEST® had a median increase in peak urinary NGAL concentration within 48 hours of 1.8 ng/ml compared with an increase of 26.2 ng/ml in the placebo arm (p=0.04). This corresponds to a clear difference in the median percentage change in the peak urinary NGAL level within 48 hours of 11.3% in the RUCONEST® arm and 205.2% in the placebo arm (p=0.001).

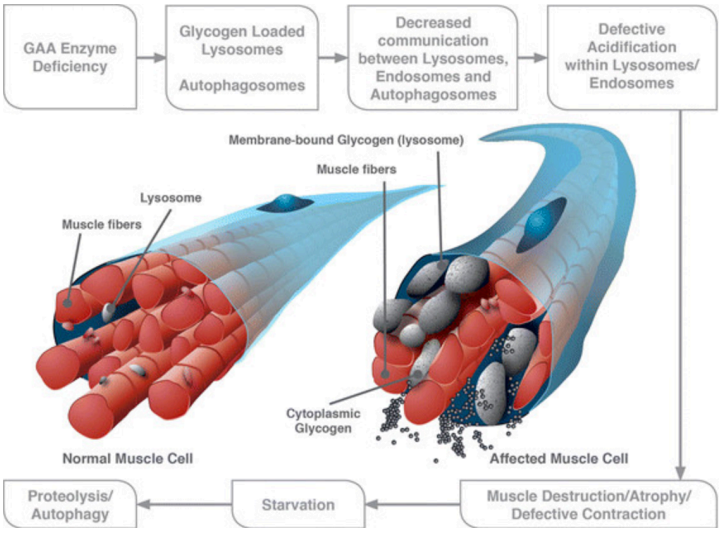
The overall assessment of the study also showed trends that patients undergoing more invasive interventions and procedures requiring higher volumes of contrast medium experienced a stronger benefit from the RUCONEST® treatment. The treatment also showed an excellent safety profile comparable to the placebo group – a particularly significant observation considering the high-risk patient group included in the study (average age approximately 77 years, with multiple comorbidities and impaired kidney function). This data, therefore, supports additional clinical investigations for the use of rhC1INH in a new indication where there is a significant unmet medical need. As a follow-up, Pharming is expected to initiate a formal Phase II study in CIN very soon.

Alpha-glucosidase in Pompe disease

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 (metabolize) 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.

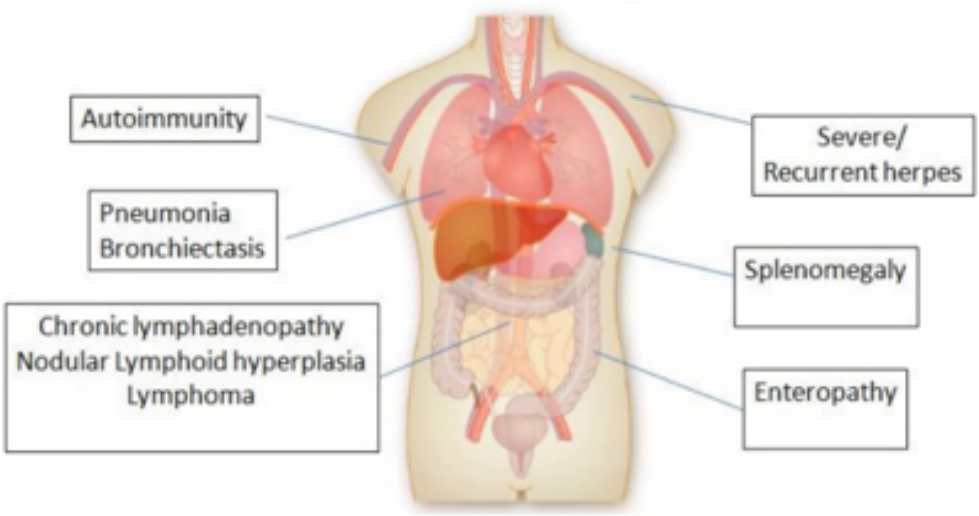
Human recombinant α-glucosidase has been produced in transgenic animals before. Until 2002, Genzyme together with Pharming generated transgenic rabbits producing α-glucosidase. Production levels at the time were as high as 8 g/L (Bijvoet et al. 1998, 1999). The transgenic material was shown to be active 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 2019, sales of Myozyme®/Lumizyme® were EUR 918 million, an increase of 8.3%. On this basis, assuming a similar growth for the products in 2020, 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 2021. We believe that, considering the

existing safety data, this trial can be concluded within 12 months, followed by a Phase II/III trial in 2022.

Leniolisib in Activated Phosphoinositide 3-kinase Delta Syndrome (APDS)

In August 2019, Pharming announced it has entered into a development collaboration and license agreement with Novartis to develop and commercialize leniolisib (CDZ173), a small molecule phosphoinositide 3-kinase delta (PI3Kδ) inhibitor being developed by Novartis to treat patients with Activated Phosphoinositide 3-kinase Delta Syndrome ("APDS"). APDS is a relatively recent recognized primary immunodeficiency disease (PID). Primary immunodeficiencies are disorders in which part of the body's immune system is missing or does not function normally. To be considered a PID, the cause of the immune deficiency must not be secondary in nature (i.e., caused by other disease, drug treatment, or environmental exposure to toxins). Most primary immunodeficiencies are genetic disorders; the majority are diagnosed in children under the age of one, although milder forms may not be recognized until adulthood. While there are over 300 recognized PIDs, most are very rare.

APDS is a PID caused by a mutation in the PIK3CD gene that increases activity of PI3Kδ, a promoter of activity in the immune system. As a result of this over-activity, the cells involved in immune response can fail to be differentiated properly, which means that sufferers are unable to react well to infections, and can suffer early cell death. Patients frequently suffer a functional inability to fight off infections, as well as developing airway and other lesions and certain cancers. Patients with APDS have increased risk of lymphoma. Both Hodgkin and Non-Hodgkin lymphoma have been reported in these patients. The incidence of lymphoma reported is as high as 13% and many of them had underlying Epstein-Barr virus infection.



Clinical features in patients with APDS

APDS is an ultra-rare disease with incidence rates across the world of approximately 1-2 per million. Importantly, there is a commercially available genetic test that can identify the patients who will benefit from CDZ173 making this program personalized medicine for these APDS patients and their family members who also have the mutation. The presence of this test is an important advantage that already have led to higher number of known patients. We therefore believe that the prevalence is considerably higher. Screening in subsets of PID patients has found rates of 1-9%. In our valuation model we estimate that 1% of all PID patients suffer from APDS.

Novartis has completed all the preclinical and clinical work to date and will continue to run the ongoing registration-enabling trial and the ongoing open label extension study. A clinical trial with 6 APDS patients was conducted as a 12-week, open-label, multisite, within-subject, dose-escalation study of oral leniolisib to assess safety, pharmacokinetics, and effects on lymphoproliferation and immune dysregulation. After 12 weeks of treatment, all patients showed amelioration of lymphoproliferation with lymph node sizes and spleen volumes reduced by 39%

(mean; range, 26%-57%) and 40% (mean; range, 13%-65%), respectively. Thus, leniolisib was well tolerated and improved laboratory and clinical parameters in APDS, supporting the specific inhibition of PI3K**δ** as a promising new targeted therapy in APDS and other diseases characterized by overactivation of the PI3K**δ** pathway.

Pharming will work alongside Novartis to complete enrollment of the ongoing trial which is expected to be completed in June 2021. Upon approval, Pharming will commercialize leniolisib through its existing commercial infrastructure in the US and Europe and look for ways to make the drug available in other markets worldwide. The Company expects that the drug can reach the market by mid 2022.

Potential acquisitions in the near future

With a relatively large cash position following the placement of EUR 125 million senior unsecured convertible bonds, Pharming has additional space to make acquisitions that will strengthen its late-stage pipeline. It already did so with acquiring the exclusive license agreement of leniolisib from Novartis. In that light, we feel that making a larger acquisition would much quicker enhance the company's potential revenue and profit in the short term. US-based Biocryst (NASDAQ: BCRX) is awaiting FDA approval for berotralstat for prophylactic use in HAE (PDUFA date 3 December 2020). In our view, Biocryst would be an ideal M&A candidate for Pharming due to:

- Access to an orally-administered drug for prophylactic use in HAE
- Gaining market share in HAE market for prophylactic use
- Synergies in the use of existing commercial infrastructure in the US
- Increased near term profitability

Financials

In 2020Q1, net product sales increased by 40% to EUR 49.3 million compared to EUR 35.2 million in 2019Q1. US net product sales increased to EUR 47.5 million (Q1 2019: €33.7 million), an increase of 41% compared to the same period last year and an increase of 8.7% compared to the 2019Q4. This strong increase is due to the continued growth in new patients using RUCONEST® (recombinant human C1 inhibitor). We feel that the COVID-19 pandemic had a positive impact as patients would want to have enough products at their disposal to be on the safe side. In Europe and the rest of the world, product sales increased to EUR 1.8 million (Q1 2019Q1: EUR 1.3 million), an increase of 38% compared to the same period last year, following the reacquisition of RUCONEST®-licensed territories per 01 January 2020.

Operating profit amounted to EUR 19.4 million from EUR 12.2 million in 2019Q1 Net profit increased by 25% to EUR 8.4 million, compared to EUR 6.7 million for 2019Q1, despite significant financial expenses of EUR 7.0 million, mainly driven by EUR 3.7 million one-off costs, associated with the pay-off of the Orbimed loan in early January, an increase of the contingent consideration of EUR 1.2 million for the final USD 25 million milestone to Bausch Health and foreign currency losses of EUR 1.1 million. The interest payments on the EUR 125 million 2020-2025 convertible bonds only amounted to EUR 0.8 million of these financial expenses, compared to the EUR 3.3 million interest for the Orbimed loan that was paid during 2019Q1 reflecting the significant decrease in financing costs going forward.

Substantial investments in its pipeline and production capacity RUCONEST®

Furthermore, the Company continued to invest in expanding the pipeline for RUCONEST®. In June 2019, it started a Phase I/II clinical study in patients with pre-eclampsia in The Netherlands. The results are expected to be available early 2021. To meet the increasing demand for

RUCONEST® from the additional programs as well as from more patients with HAE, Pharming has invested in expanding its production capacity. In January, Pharming announced it had received European Medicines Agency (EMA) approval of a Type II Variation for a new production facility for RUCONEST®. With the addition of this new facility, Pharming will significantly increase the production capacity of RUCONEST® as it becomes fully operational over the coming year. Pharming is now able to release the product that was manufactured at the facility during the approval process for commercialization in the EU. As previously announced, Pharming had identified a potential risk of short-term pressure on the supply of RUCONEST® for the European market due to increasing demand for the product. With the approval of this new facility, the Company believes the risk to supply is now significantly reduced. The new facility's post-approval supplement (PAS) for the distribution of RUCONEST® in the US is still under review by the Food and Drug Administration (FDA). Approval of the new facility for delivery in the US is expected in 2020H1.

In August 2019, the Company announced that it had entered into a development collaboration and license agreement with Novartis to develop and commercialize leniolisib (CDZ173), a small molecule phosphoinositide 3-kinase delta (PI3Kδ) inhibitor being developed by Novartis to treat patients with APDS. Novartis and Pharming will continue the development of the compound through its current registration-enabling trial in partnership. Pharming will commercialize the treatment if it obtains approval from regulators. Pharming paid an upfront amount of EUR 17.9 million (USD 20 million) for the license. Although further details of the terms were not disclosed, we believe that Novartis will be eligible to receive tiered double-digit royalties from Pharming once the drug is approved.

Cash Position Continues to Grow, Financial Position Strengthened

Positive cashflows during the quarter were driven by strong revenue despite intensified competition, generating more than EUR 19 million of cash above the cash required for operating

costs. This was then reduced, mainly, by payment of EUR 5.5 million of the total EUR 7.5 million payable to Sobi for reacquisition of the EU commercialization rights to RUCONEST® and the USD 20 million payment (EUR 18.1 million) for the penultimate sales performance milestone paid to Bausch Health Companies Inc. These payments and the balance of the repayment in full of the remaining Orbimed loan facility and the associated penalties for early repayment (totalling to EUR 49.7 million) and the net proceeds of the EUR 125 million convertible bonds minus interest payment, resulted in an increase in the cash position of EUR 67.5 million to EUR 136.1 million at 31 March 2020 (EUR 68.6 million at 31 December 2019).

For 2020FY, we expect revenues to come in at EUR 215 million, an increase of 27%. Although the Company showed very strong figures in 2020Q1, we are still a bit cautious to see if Pharming can maintain this growth for the rest of the year. Seasonal patterns are in order. Nonetheless we feel that bottom line the Company has room for considerable improvement with relatively lower operating costs and much lower financial expenses.

Profit & Loss Statement

EUR million	2020Q1	2019Q1	2018A	2019A	2020E	2021E
Total Revenues	49.3	35.2	135.1	169.0	215.0	287.5
Cost of Sales	(5.4)	(5.4)	(22.1)	(21.4)	(25.7)	(34.4)
Gross Profit	**43.9**	**29.8**	**113.0**	**147.7**	**189.3**	**253.1**
R&D Costs	(8.0)	(5.3)	(28.9)	(32.9)	(37.9)	(38.7)
G&A Costs	(5.2)	(3.0)	(12.2)	(14.3)	(16.0)	(17.0)
Marketing& Sales	(11.5)	(9.6)	(34.5)	(39.9)	(50.3)	(65.9)
Operating Profit	**19.4**	**12.2**	**38.0**	**60.9**	**85.1**	**131.5**
Financial Income/(Expenses)	(7.0)	(2.5)	(37.1)	(14.5	(10.0)	(10.0)
Tax credit/(expense)	**(4.0)**	**(3.0)**	**24.1**	**(10.5)**	**(18.0)**	**(25.0)**
Net Profit/(Loss)	**8.4**	**6.7**	**25.0**	**36.2**	**57.1**	**96.5**

Consolidated statement of cash flows

EUR million	March 31st 2019	March 31st 2020	Dec 31st 2018A (12 months)	Dec 31st 2019A (12 months)
Cash flow from operating activities	9.8	19.4	40.4	73.1
Cash flow from investing activities	(0.3)	(6.3)	(3.8)	(32.7)
Cash flow from financing activities	(27.9)	54.8	(18.0)	(54.7)
Cash and cash equivalents at beginning of the period	81.5	68.6	60.0	81.5
Net change in cash and cash equivalents	(18.4)	67.9	21.5	(12.9)
Cash and cash equivalents at end of the period	66.5	136.1	81.5	68.6

Consolidated Balance sheet

EUR million	2020Q1	Dec 31st 2018A (12 months)	Dec 31st 2019A (12 months)
Non current assets	125.0	99.1	121.7
Current Assets	45.1	35.1	41.3
Cash and cash equivalents	133.8	80.3	66.3
Total assets	303.9	214.5	228.2
Equity	115.6	61.7	104.7
Non current liabilities	143.9	70.2	21.6
Current liabilities	44.4	82.6	101.9
Total equity and liabilities	303.9	214.5	228.2

Valuation

Valuation Revised Upwards to EUR 2.84

Based on our NPV based valuation, we believe that Pharming is substantially undervalued at the current share price of EUR 1.35. We have increased our valuation for the company taking into account new programs for RUCONEST® and its exclusive license of leniolisib (acquired from Novartis August 2019). The company's current total value should, therefore, increase from EUR 2.0 billion million to EUR 2.1 billion, which translates, based on the fully diluted number of shares of approximately 742 million, into EUR 2.84 per share.

Valuation RUCONEST in Acute HAE

In estimating a value for RUCONEST®, we took into account potential markets in the US and Europe, with the US market calculated to be 75-85% of the total market. With the buyback of the commercial rights of RUCONEST in all remaining EU markets from Sobi, we estimate that revenues from RUCONEST in the EU will increase substantially. We calculate a Risk-adjusted Discount Rate of 15%. 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% should be possible. Furthermore, we expect that leniolisib will be on the market in 2022.

Year	2020	2021	2022	2023	2024	2025	2026	2027	2028	2029	2030
Market Size US Acute	**1212**	**1273**	**1337**	**1404**	**1474**	**1547**	**1625**	**1706**	**1791**	**1881**	1975
Penetration	17,5%	20,0%	22,5%	25,0%	26,0%	27,0	26,0%	25,0%	25,0%	24,0	**23.0**
Total Revenue US & EU	**206.1**	**275.5**	**346.2**	**405.3**	**446.4**	**489.8**	**494.0**	**497.5**	**518.2**	**521.1**	**523.0**
Margin up to 65%	62.1	96.5	210.3	245.8	269.6	295.0	297.8	300.3	314.0	316.1	317.6
WACC 15%	0.87	0.76	0.66	0.57	0.50	0.43	0.38	0.33	0.28	0.25	0.21

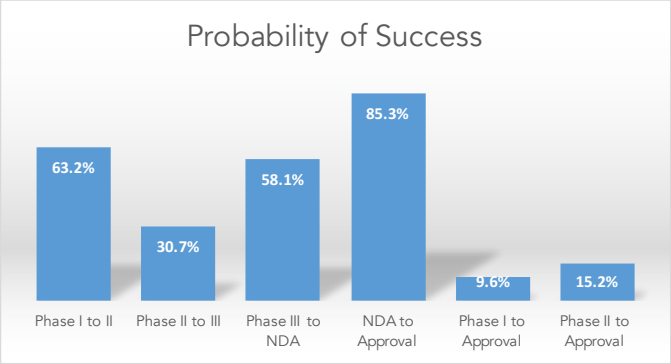
NPV (million)	54.0	57.8	121.8	140.5	134.0	127.5	112.0	98.2	89.3	78.1	68.3
Total NPV (million)											1,267.6
Value per share (EUR)											1.71

Phase Success and Likelihood of Approval (LOA)

In estimating a value for the new clinical programs with RUCONEST® in AKI and Pre-Eclampsia, the Pompe program and leniolisib, 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 changes in the Biomedtracker database were analyzed. A phase change is a 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 most extensive 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.

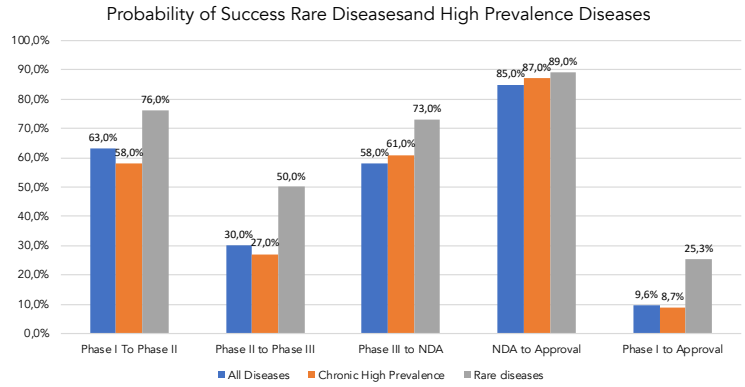
The Phase I transition success rate was 63.2% (n=3,582). As this Phase is typically conducted for safety testing and is not dependent on efficacy results for candidates to advance, it is common for this Phase to have the highest success rate among the clinical phases across most categories analyzed in this report. Phase I success rates may also benefit from delayed reporting bias, as some more substantial 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 weakest 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 significant, 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 most protracted 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 US 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 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 disorders and 3x more elevated than the 8.7% LOA for chronic, high prevalence diseases.



Source: BIO Industry Analysis

Valuation RUCONEST in Pre-Eclampsia

For the valuation of RUCONEST® in Pre-Eclampsia we made the following assumptions. With initiating a first Phase I/II last year, we believe that market introduction is possible in 2025/26. Based on research from Europe PMC and from the US National Center for Health, we went with incidence rates of 2.3% and 3.2% of the total number of births in the EU and US respectively. Of that number we went with an addressable market of 60%. We priced the product at EUR 35,000 per treatment. Based on the report from BIO, we worked with a LOA of 35%. The discount rate was calculated at 15%. This leads to a total current value of the RUCONEST® program in Pre-Eclampsia of EUR 122 million or EUR 0.16 per share.

Year	2025	2026	2027	2028	2029	2030	2031	2032	2033	2034	2035
Market Size Pre eclampsia (EUR m)	5188	5227	5266	5306	5345	5385	5426	5467	5508	5549	5590
Penetration	0,5%	1,0%	2,0%	4,0%	6,0%	7,0%	8,0%	9,0%	10,0%	10,0%	9,0%
Total Revenue US&EU (EUR m	12.6	38.9	78.3	157.8	265.9	349.3	406.2	463.9	522.5	554.9	531.8
Margin up to 60%	7.6	23.3	47.0	94.7	159.5	209.6	243.7	278.4	313.5	332.9	319.1
WACC 15%	0.43	0.38	0.33	0.28	0.25	0.21	0.19	0.16	0.14	0.12	0.11
NPV (million)	3.3	8.8	15.4	26.9	39.4	45.1	45.6	45.2	44.3	40.9	34.1
Total NPV (million) EUR											**122.1**
Value per share (EUR)											**0.16**

Valuation RUCONEST in Acute Kidney Injury (AKI)

For the valuation of RUCONEST® in AKI we made several assumptions. Assuming the start of a new Phase II in 2020H2, we believe that market introduction is possible in 2024. 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 25 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 15,000 per treatment in the US and USD 12,000 in the EU. Based on the report from BIO, we worked with a LOA of 40%. The discount rate was calculated at 15%. This leads to a total current value of the RUCONEST® program in AKI of EUR 174 million or EUR 0.23 per share.

Year	2024	2025	2026	2027	2028	2029	2030	2031	2032	2033	2034
Market Size CIN (EUR m)	2576	2584	2593	2601	2610	2618	2627	2636	2644	2653	2662
Penetration	1,0%	2,0%	3,5%	5,0%	7,0%	10,0%	12,5%	15,0%	17,5%	20,0%	22,5%
Total Revenue US&EU (EUR m	25.8	51.7	90.7	130.1	182.7	261.8	328.4	395.3	462.8	530.6	598.9
Margin up to 65%	16.7	33.6	59.0	84.5	118.7	170.2	213.4	257.0	300.8	344.9	389.3
WACC 15%	0.50	0.43	0.38	0.33	0.28	0.25	0.21	0.19	0.16	0.14	0.12
NPV (million)	8.3	14.5	22.2	27.6	33.8	42.1	45.9	48.0	48.9	48.7	47.8
Total NPV (million) EUR											**173.7**
Value per share (EUR)											**0.23**

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 2021, we believe that market introduction is possible in 2025. The prevalence of Pompe's Disease is roughly 1 in 50,000 persons. We calculate an addressable market of 40% in the EU and 80% in the US with a peak market share of 25%, which is rather conservative. We priced the product at EUR 300,000 per treatment in the US and EUR

240,000 in the EU. Based on the report from BIO, we worked with a LOA of 35%. The discount rate was calculated at 15%. This leads to a total current value of the alpha-glucosidase program in Pompe's Disease of EUR 180 million or EUR 0.24 per share.

Year	2025	2026	2027	2028	2029	2030	2031	2032	2033	2034	2035
Patients Pompe's Disease	18140	18240	18340	18440	18540	18640	18740	18840	18940	19040	19140
Penetration	2,0%	4,0%	8,0%	12,0%	15,0%	18,0%	22,0%	23,0%	24,0%	25,0%	25,0%
Total Revenue US&EU	56.7	114.1	229.6	346.3	435.5	525.6	646.1	679.3	712.9	746.8	751.0
Margin up to 65%	36.9	74.2	149.2	225.1	283.0	341.6	419.9	441.5	463.4	485.4	488.1
WACC 12%	0.43	0.38	0.33	0.28	0.25	0.21	0.19	0.16	0.14	0.12	0.11
NPV (million)	15.9	27.9	48.8	64.0	70.0	73.4	78.5	71.8	65.5	59.7	52.2
Total NPV (million) EUR											**180.5**
Value per share (EUR)											**0.24**

Valuation leniolisib in APDS

For the valuation of leniolisib in APDS we made several assumptions. The pivotal Phase III trial is currently ongoing and the Company expects that market introduction is feasible in 2022. Based on market research that estimate that 1 in 1200 suffer from some kind of PID. Screening in subsets of PID patients learned that 1-9% of PID patients have APDS. This boils down to at least 7,000 patients in the EU and the US. We priced the product at EUR 250,000 per treatment in the US and EUR 200,000 in the EU. Based on the report from BIO, we worked with a LOA of 60%. The discount rate was calculated at 12%. This leads to a total current value of leniolisib in APDS of EUR 361 million or EUR 0.49 per share.

Year	2022	2023	2024	2025	2026	2027	2028	2029	2030	2031	2032
Patients APDS	7039	7055	7071	7087	7103	7120	7136	7152	7169	7185	7202
Penetration	1%	2%	4%	7,5%	10%	13%	15%	17,5%	20%	22,5%	25%
Total Revenue US&EU	7,5	29,9	60,0	112,8	150,8	189,0	227,4	266,0	304,8	343,8	383,0
Margin up to 65%	4,9	19,5	39,0	73,3	98,0	122,9	147,8	172,9	198,1	223,5	249,0
WACC 12%	0,71	0,64	0,57	0,51	0,45	0,40	0,36	0,32	0,29	0,26	0,23
NPV (million)	3,5	12,4	22,1	37,2	44,3	49,6	53,3	55,7	57,0	57,4	57,1
Total NPV (million) EUR											**361.2**
Value per share (EUR)											**0.49**

Analyst: Marcel Wijma MSc

Marcel Wijma, Chief Research Officer and managing partner, has a longstanding history in financial biotech research. After selling Van Leeuwenhoeck 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.