Pharming Group N.V.

Full Year 2021 Financial Results Analyst Call

March 17, 2022

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

Forward looking statements

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Speakers

Anurag Relan
Chief Medical Officer

Sijmen de Vries
Chief Executive Officer

Jeroen Wakkerman
Chief Financial Officer

Q&A

Execution delivering future growth: Investment Summary

- A well-funded business supported by commercial sales and a growing pipeline for the treatment of rare and ultra diseases with unmet medical needs
- Lead product, RUCONEST® (rhC1INH), launched in over 40 countries with sales of over US$198.9 million in 2021 and increasing patient demand in the treatment of HAE
- Near-term inflection point with anticipated launch of leniolisib in H1 2023, for the treatment of orphan disease APDS to support further sales growth – market opportunity with an estimated >1,350 patients (500 US, 675 EU, 190 Japan) living with APDS and more than 350 patients already identified by Pharming
- Established specialist commercial infrastructure across US and Europe- able to leverage for in-licensed products to bring new/specialist products to market
- Leveraging in-house expertise to drive R&D of specialist products, including in-licensed potentially curative gene therapy candidate for HAE, OTL-105
- Opportunity to further investigate lifecycle management potential of internal portfolio in the treatment of new indications with unmet need
- Experienced leadership team and strong balance sheet to support ambitious growth strategy, including further in-licensing and M&A opportunities

Three-pillar objectives to build a fully integrated sustainable business

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Grow our global fully integrated commercial infrastructure

Continuing to grow RUCONEST® sales through further country launches & extending HAE portfolio

- Fully commercialize RUCONEST® in all major international markets with our own sales forces
- Commercialize leniolisib for APDS and future products in all major markets

Near-term expansion of portfolio within our rare/ultra-rare in-house expertise to grow our business

In-licencing/ acquisition of late-stage assets in rare/ultra-rare diseases

- Developing rhC1INH and PI3Kδ in follow on indications with unmet medical need
- Leverage genetic testing capability to identify additional late-stage/ultra-rare disease market opportunities

Long-term identification and development of solutions for patients with unmet medical needs

Grow our rare/ultra-rare patient identification database with genetic testing

- Development of early stage OTL-105, an ex-vivo HSC gene therapy candidate for HAE
- Development of early-stage asset, rhaGLU, an enzyme replacement therapy for Pompe disease

Strategy in action: Progress during 2021

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Commercial: RUCONEST®

- Expanding reach of RUCONEST® with commercialization agreement with NewBridge Pharmaceuticals in North Africa and the Middle East
- RUCONEST® reimbursement in Spain
- Renewed strategic manufacturing agreement with Sanofi

Late-stage pipeline

- Positive top-line data in the pivotal Phase II/III study of leniolisib for the treatment of activated PI3Kδ syndrome
- Significant investment in pre-launch activities for leniolisib; anticipated global regulatory filings Q2 2022
- Launch of navigateAPDS a sponsored genetic testing program to identify patients with APDS
- Received positive decision from the EMA on the Paediatric Investigation Plan (PIP) for leniolisib in Europe

Earlier-stage pipeline

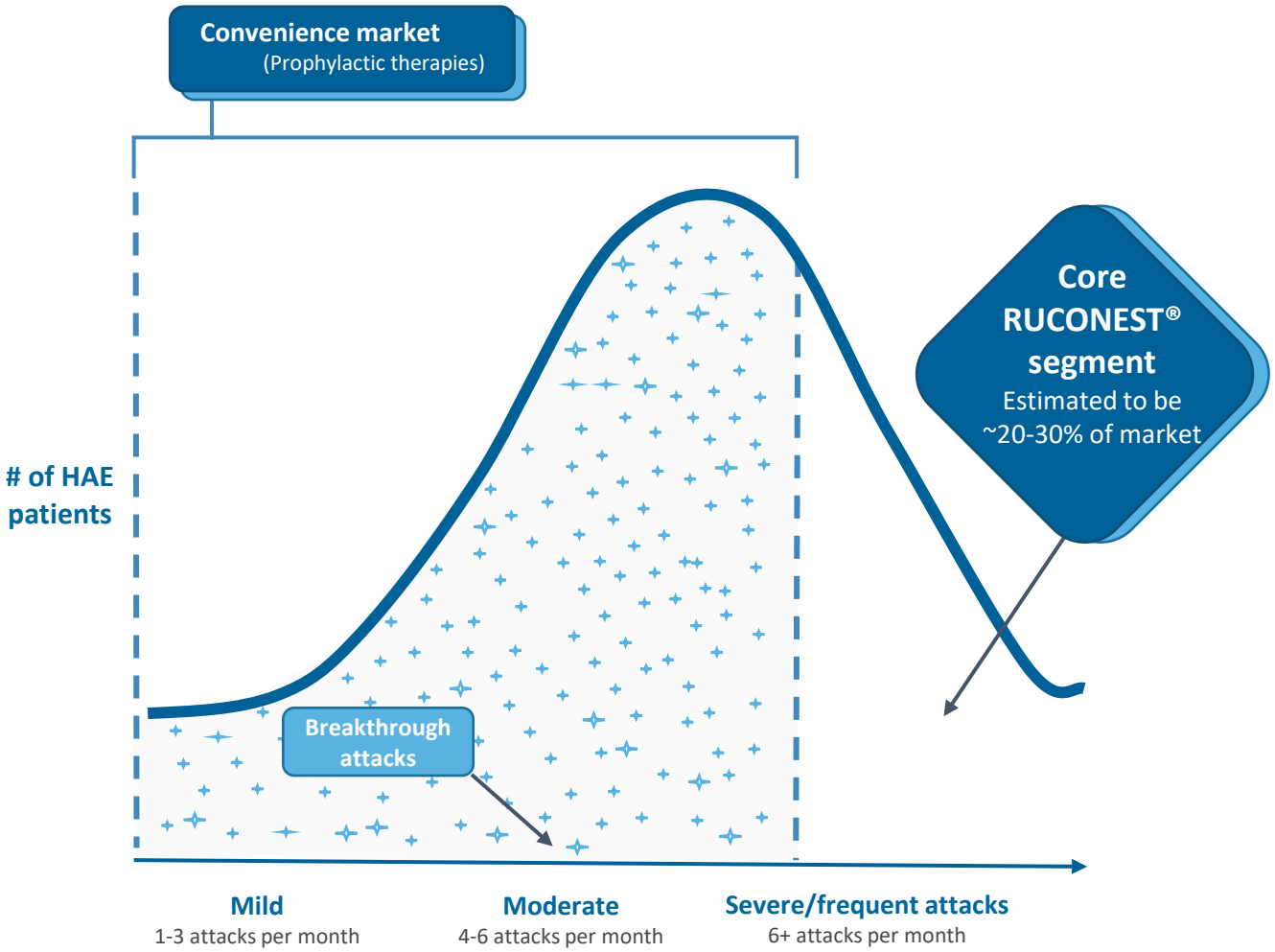
- In-licensing of OTL-105, a potentially curative candidate for HAE from Orchard Therapeutics
- First patient enrolled in Phase IIb study for rhC1INH in the prevention of Acute Kidney Injury
- Topline results from rhC1INH clinical trial in severe pneumonia as a result of COVID-19 infection

HAE & RUCONEST®

Ongoing strong sales performance supporting future investment in long-term growth

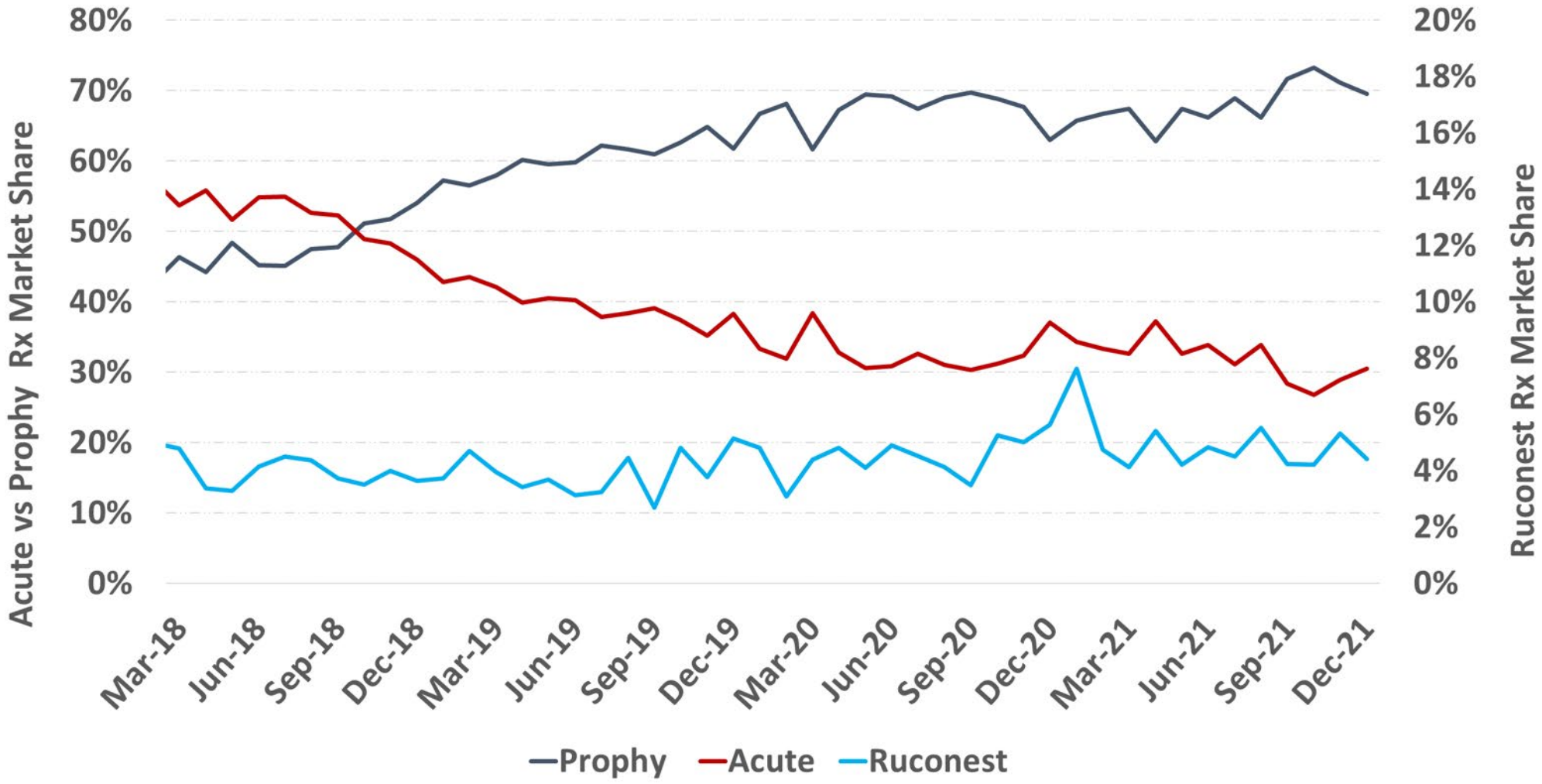
RUCONEST® positioning in the treatment of HAE

- HAE is caused by a deficiency of C1-INH, resulting in attacks of severe swelling (angioedema) in various parts of the body
- Patients use medication for treatment and prevention (prophylaxis) of attacks
- RUCONEST® approved for the treatment of acute HAE in adults and adolescents in the US and the EU
- Increasing use of prophylaxis because patients want to be attack-free
  - New treatments offer better attack reduction rates than previous IV plasma-derived C1-INH prophylaxis treatment
  - Although kallikrein/bradykinin inhibitors block the main pathway for symptomatology, C1-INH levels remain low
  - Approx. half of patients using new prophylaxis treatments continue to have breakthrough attacks, some frequently, and regularly use acute medication
- Therefore, with a continued need for safe and reliable acute treatments, we remain confident in the ongoing demand for RUCONEST®



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

Ongoing demand for acute therapy following stabilization of prophylactic market



Source: *Payer sourced Claims through Dec '21.*

Based on partial payer data, that we estimate represents ~25% of the market

APDS & leniolisib

Expanding our commercial portfolio and leveraging our existing infrastructure to drive growth

Significant unmet need in APDS (activated PI3Kδ syndrome)

Burden of APDS[1-4]

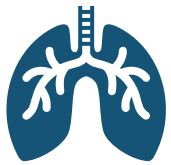
- Estimated >1,350 patients (500 US, 675 EU, 190 Japan) live with APDS
  - More than 350 patients already identified by Pharming
  - Greater understanding of PIDs is revealing a larger patient population[5]
- Years spent undiagnosed or misdiagnosed, seeing 4-5 specialists
- Symptoms begin in childhood & disrupt school and social development
- Significant impact on QoL:
  - Surgical interventions are common
  - Care typically managed by >4 doctors
  - Depression and fatigue significantly impact QoL

Current treatment options for APDS[6]

- Supportive/non-specific therapies (e.g. antibiotics, steroids)
- Immunoglobulin replacement therapy (IRT) infusions
- mTOR inhibitors (e.g., sirolimus, rapamycin) off-label for lymphoproliferative symptoms only

Often used together

- Hematopoietic stem cell transplantation
- No approved therapy for treatment



Severe infections, permanent lung damage

Severe GI disease

Severe swollen lymph nodes, spleen and liver

Autoimmunity including severe anemias

13-28% of patients develop lymphoma

1. DOF, Pharming Healthcare, Inc. 2021. 2. Jamee M, et al. Clin Rev Allergy Immunol. 2019;May 21. 3. Maccari ME, et al. Front Immunol. 2018;9:543. 4. Carpier JM, Lucas CL. Front Immunol. 2018;8:2005. 5. Chan AY, et al. Front Immunol. 2020;11:239.; IUIS: International Union of Immunological Societies. 6. Coulter TI, Cant AJ. Front Immunol. 2018;9:2043. 6. Bousfiha A, et al. J Clin Immunol. 2020;40(1):66-81.

Uncovering “APDS”: US targeted patient identification strategy

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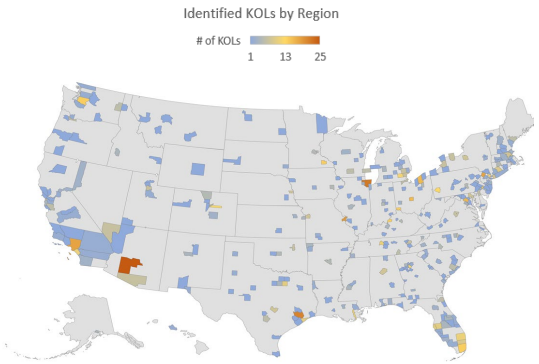
1.

BUILD APDS NETWORK



The US has created a KOL network & referral pathway of prescribers actively supported by field medical & diagnostic liaisons

2.

ANALYTICS & AI

Patient identification using sophisticated & targeted digital strategy & A.I

3.

OUTREACH & EDUCATION

Genetic Testing
navigateAPDS
by Pharming

Immune Deficiency Foundation
Global Genes
Jeffrey Modell Foundation

‘Free of charge’ genetic testing, supported by strong community connections and social media advocacy

Leniolisib in development for the treatment of APDS

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US PDUFA target date

APDS : Activated PI3K Delta Syndrome — **Q1/2023**

leniolisib[1,2]

- Effective oral selective PI3Kδ inhibitor
- Precision biomarker response demonstrates impact on root cause
- Potential to mitigate progression of disease & reduce treatment burden
- APDS diagnosis made by a commercially available genetic test[3]
- Orphan drug designation granted by US FDA and European Commission
- Able to leverage Pharming's existing commercial infrastructure

1. Rao VK, et al. Blood. 2017;130(21):2307-2316. 2. Hoegenauer K, et al. ACS Med Chem Lett. 2017;8(9):975-980. 3. Chinn IK, et al. J Allergy Clin Immunol. 2020;145(1):46-69. 4. Okkenhaug K, Vanhaesebroeck B. Nat Rev Immunol. 2003;3:317-330. 5. Fruman DA, et al. Cell. 2017;170(4):605-635.

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

Pivotal trial design[1,2]

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Part 1
Dose-finding

12 weeks
N=6

Leniolisib
10, 30 and 70 mg BID

- Non-randomized, open-label, dose-escalation study
- Population: Adults with APDS-associated mutation in the PI3Kδ gene (p110δ, i.e. PIK3CD), lymphoproliferation and APDS-typical clinical manifestations/history
- Primary outcomes: Safety & tolerability, PK/PD, pAKT inhibition
- Oral dose 70 mg BID selected for part 2

Part 2 –
Placebo-controlled

Randomized period
12 weeks
N=31

Leniolisib 2:1
70 mg BID

Placebo

- Randomized, triple-blinded (patient, caregiver, investigator), placebo-controlled, fixed-dose study
- Co-primary efficacy endpoints (lymphadenopathy and immunophenotype normalization)
  - Change from baseline in the index lesions selected as per from MRI/CT imaging
  - Change from baseline in percentage of naïve B cells out of total B cells
- Safety assessments

Open-label Extension Study
Leniolisib

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

1. ClinicalTrials NCT02435173. https://clinicaltrials.gov/ct2/show/NCT02435173 Accessed July 17, 2020. 2. Rao VK, et al. Blood. 2017;130(21):2307–2316.

Leniolisib APDS Part 1 data

Safety & Efficacy[1]

- Leniolisib was well tolerated at all oral doses (10 mg, 30 mg, 70 mg), but most effective at 70 mg in patients >45 kg
- No significant side effects or study drug discontinuations
- 12 weeks of leniolisib resulted in less disease activity and greater patient well-being

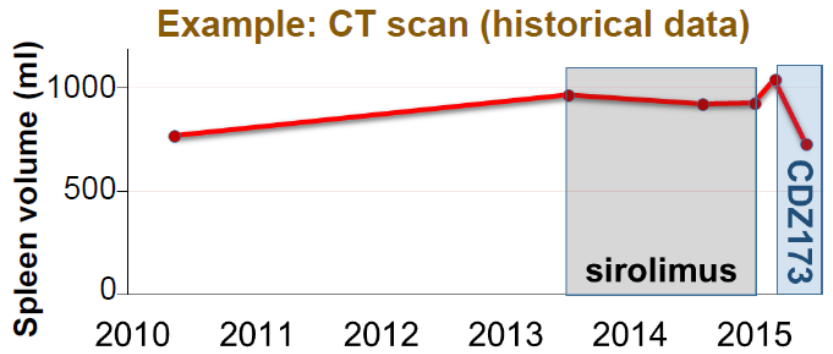
Normalization of B & T cells[1]

Suppression of PI3K/AKT pathway activity → Naïve B cells normalized; elevated transitional B cells reduced → Reduction of IgM, serum chemokines → Reduced dysfunctional and senescent T cells → Regression of the lymph nodes & spleen size

Reduction in lymph node and spleen size[1]

	Mean change from baseline ± SD (%)
Lymph node SPD	-40 ± 19
Spleen 3D volume	-39 ± 10

2

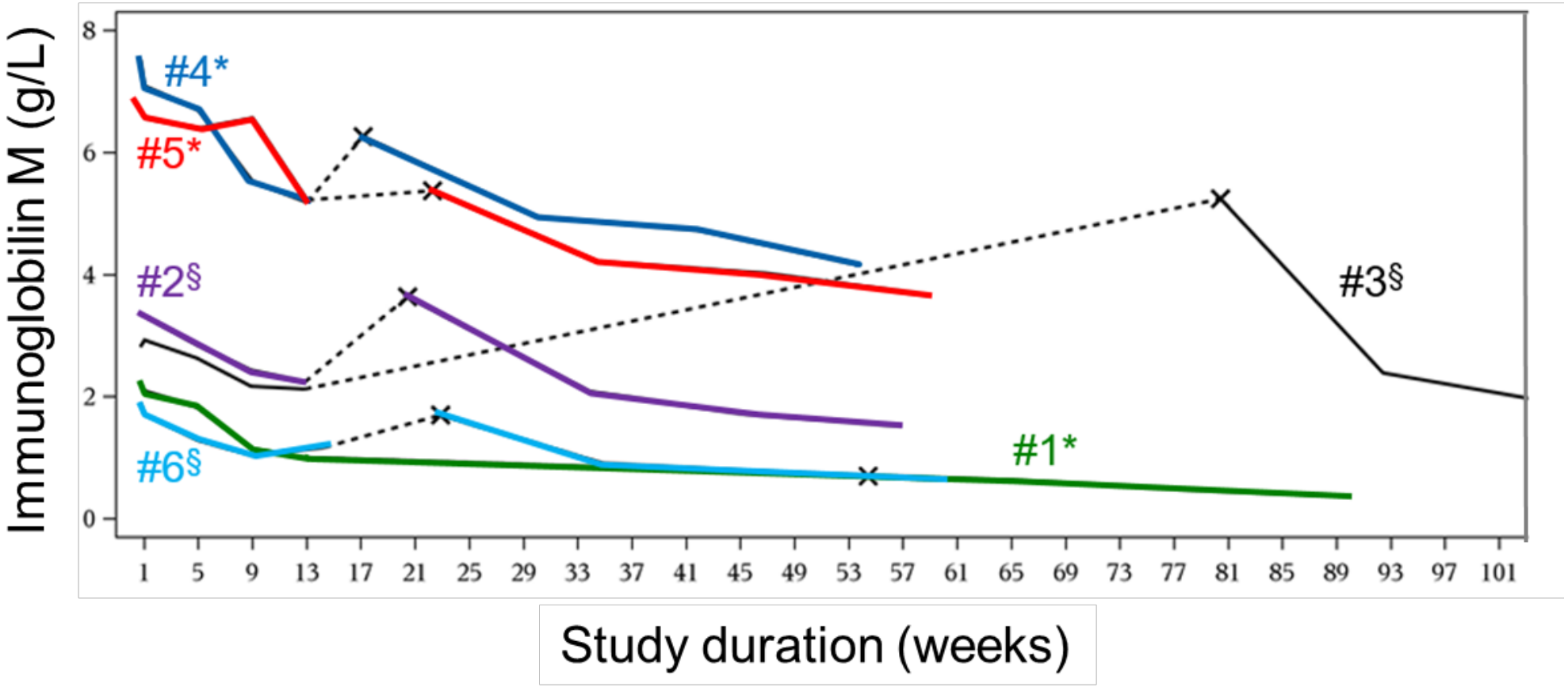


SD, standard deviation; SPD, sum of product diameters.

1. Rao VK, et al. *Blood*. 2017;130(21):2307-2316. 2. Data on file, Pharming Healthcare, Inc. 2019.

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

Long Term Leniolisib Results (N=6)



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

Slide courtesy of Dr V. Koneti Rao.
Blood (2018) 132 (Supplement 1): 3706.

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

Leniolisib APDS Part 2: Efficacy data

Primary efficacy results demonstrated clinical efficacy of leniolisib over placebo

Reduction from baseline in log10 transformed sum of product diameter in index lymphadenopathy lesions ($p=0.0012$)

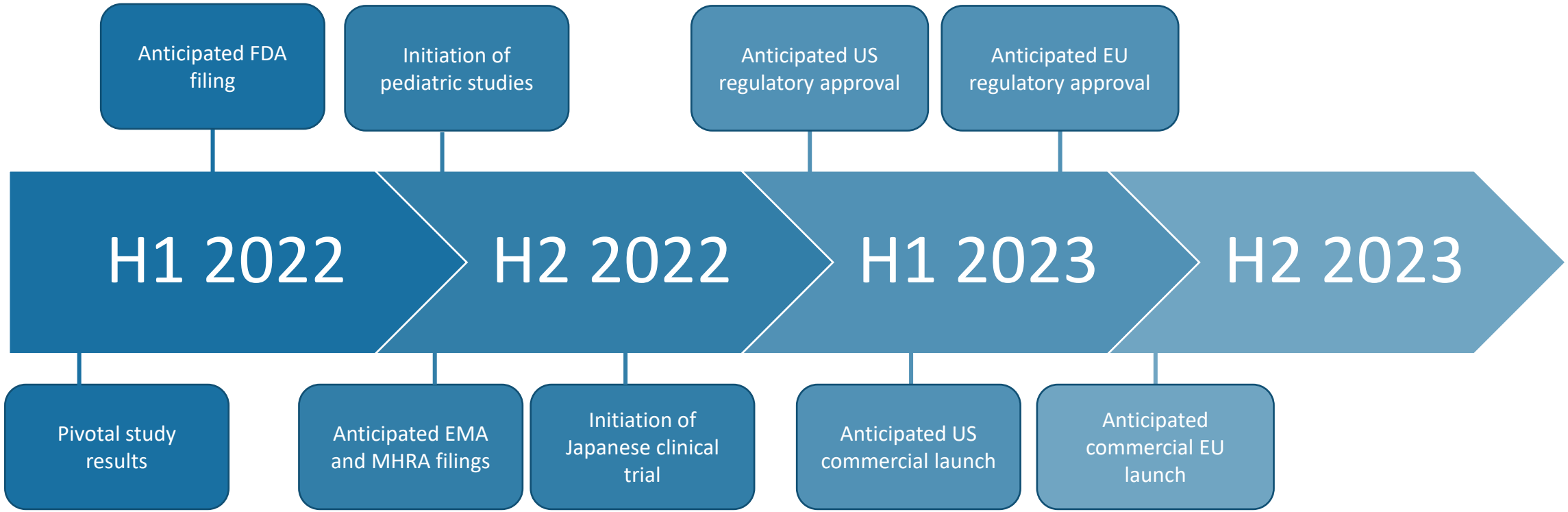
Normalization of immune dysfunction, as evidenced by increased proportion of naïve B cells from baseline ($p<0.0001$)

Full results will be presented at upcoming medical conferences and published in a peer-reviewed journal

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

Data on file, Pharming Healthcare, Inc. 2022.

Next steps: upcoming milestones*



*These dates are not an assurance of future performance; they are based on current expectations and assumptions regarding the future of our business. Please refer to our Forward-looking Statement on slide 2 of this presentation.

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

HAE & OTL-105

Grow and extend our HAE franchise

OTL-105: developing a best-in-class HAE gene therapy

- Collaboration with Orchard Therapeutics to develop and commercialize an *ex vivo* autologous hematopoietic stem cell (HSC) gene therapy for HAE
- OTL-105 inserts one or more functional copies of the SERPING1 gene into patients own HSCs *ex vivo* which are then transplanted back into the patient for potential durable C1-INH production
- In preclinical studies, to date, OTL-105 demonstrated high levels of SERPING1 gene expression via lentiviral-mediated transduction in multiple cell lines and primary human CD34+ HSCs. The program also achieved production of functional C1-INH, as measured by a clinically validated assay

Orchard therapeutics

- Expertise in HSC gene therapy
- Vector development and testing
- Established CDMO network
- Murine transplant studies
- Internal discovery capabilities

+

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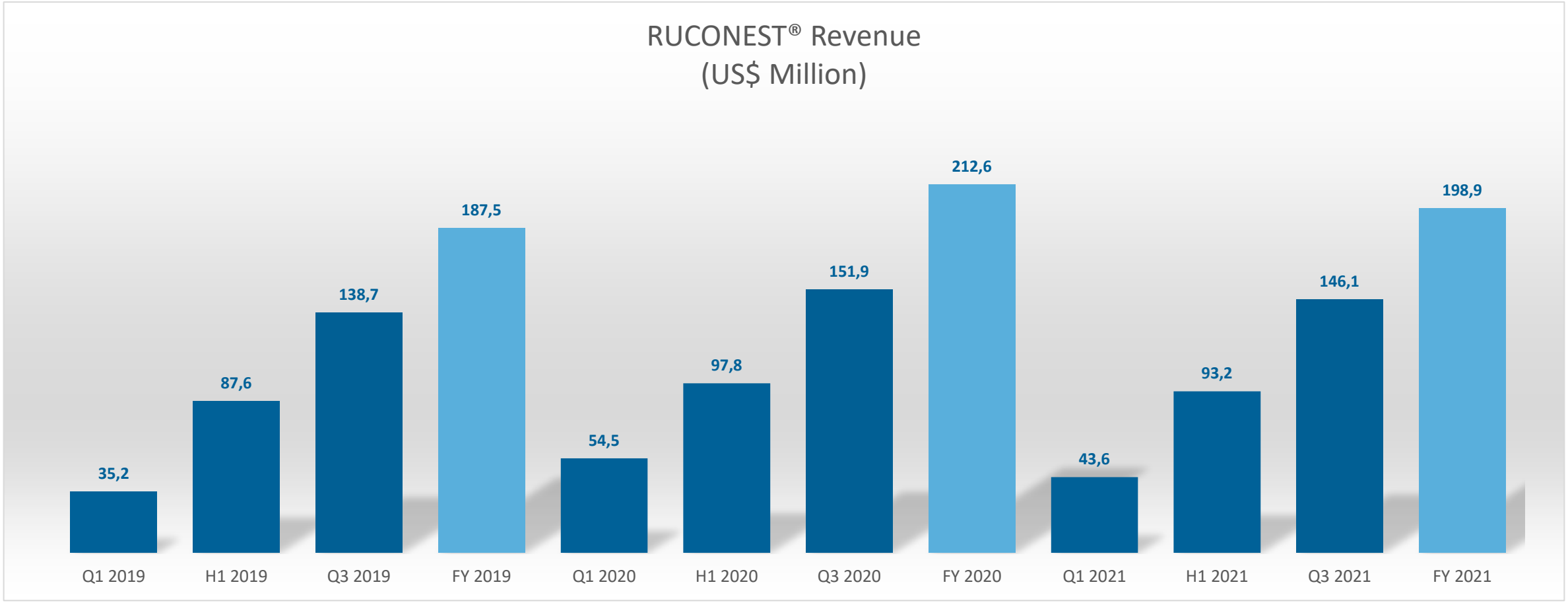
- Extensive clinical and commercial expertise in HAE
- Pre-clinical disease models for HAE
- Capital to fund ongoing development and future commercialization

Combined expertise and experience to develop a best-in-class HAE gene therapy to provide the potential for life-long prophylaxis following a single administration

Financial highlights from 2021: Building a sustainable business (1/6)

REVENUE

- Revenues for the full year of 2021 were US$198.9 million a 6% decrease from US$212.2 million in 2020.



Financial highlights from 2021: Building a sustainable business (2/6)

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REVENUE FROM SALES

- Revenues from US sales in 2021 were US$193.4 million a 5% decrease compared to 2020 (US$202.7 million).
  - Ongoing recovery in sales following the impact of COVID-19 on the US healthcare economy in Q1 2021, as previously noted in our Q1 2021, Q2 2021 and Q3 2021 financial reports.
- In 2021 Europe sales decreased to US$4.9 million, from US$8.2 million in 2020.
  - Mainly caused by phasing of ordering, as stated in the Company's Q1 2021, H1 2021 and Q3 2021 financial reports.
- Rest of World revenue (excluding Europe) decreased to US$0.5 million (from US$1.3 million in 2020).

GROSS PROFIT

- Gross profit for 2021 was US$177.7 million, a 6% decrease in comparison to 2020 (US$188.6 million), in line with the decrease in revenues.

Financial highlights from 2021: Building a sustainable business (3/6)

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OPERATING PROFIT & COST

- Operating profit of US$36.9 million in 2021, before US$23.3 million of one-off costs, relating to investment in the pipeline of US$13.1 million to in-license OTL-105 from Orchard Therapeutics and impairment of tangible and intangible assets (US$10.2 million) as result of strategic decisions.
- Operating profit after one-off costs are US$13.6 million. We continue significant investment in Pharming's long-term growth:
  - including increased R&D expenditure,
  - increased pre-launch marketing preparations and;
  - manufacturing cost for leniolisib (US$11.6 million),
  - and increased employee numbers to support growth (US$8.2 million).
  - insurance costs increased due to the Nasdaq listing (US$5.5 million).

NET PROFIT

- Net profit was US$16.0 million, a 58% decrease compared to the year 2020 (US$37.7 million), due to a significant increase in operating expenses, partly offset by favorable foreign currency effects (US$14.9 million).

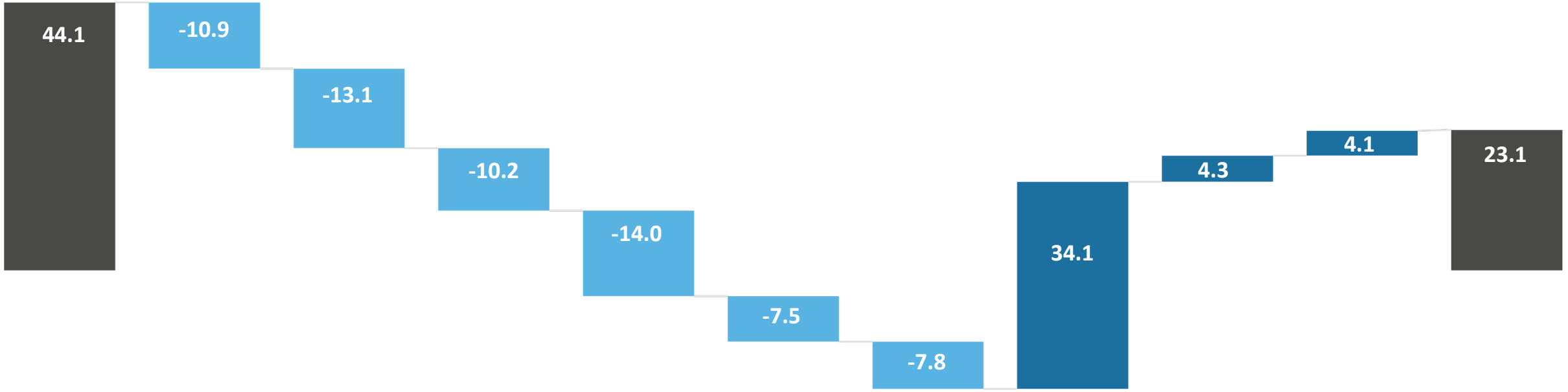
Financial highlights from 2021: Building a sustainable business (4/6)

CASH & CASH EQUIVALENTS

- Cash and cash equivalents, together with restricted cash, decreased from US$206.7 million at the end of 2020 to US$193.0 million at the end 2021,
  - Mainly due to positive cash flows from operating activities of US$37.8 million including the US$13.1 million one-off payment to Orchard Therapeutics.
  - These are offset by negative cash flows from investments and financing activities, totaling US$49.3 million.
  - This includes the payment of the final US$25.0 million milestone to Bausch Health Inc. in relation to the re-acquisition of the North American RUCONEST® commercialization rights in 2016.

Financial highlights from 2021: Profit before tax 2020 – 2021 (5/6)

(US$ Million)



Profit before tax 2020

Underlying business gross profit

OTL-105 investment

Impairment tangible and intangible assets

Increase in R&D expenditure

Increased SG&A expenditure

Increased M&S expenditure

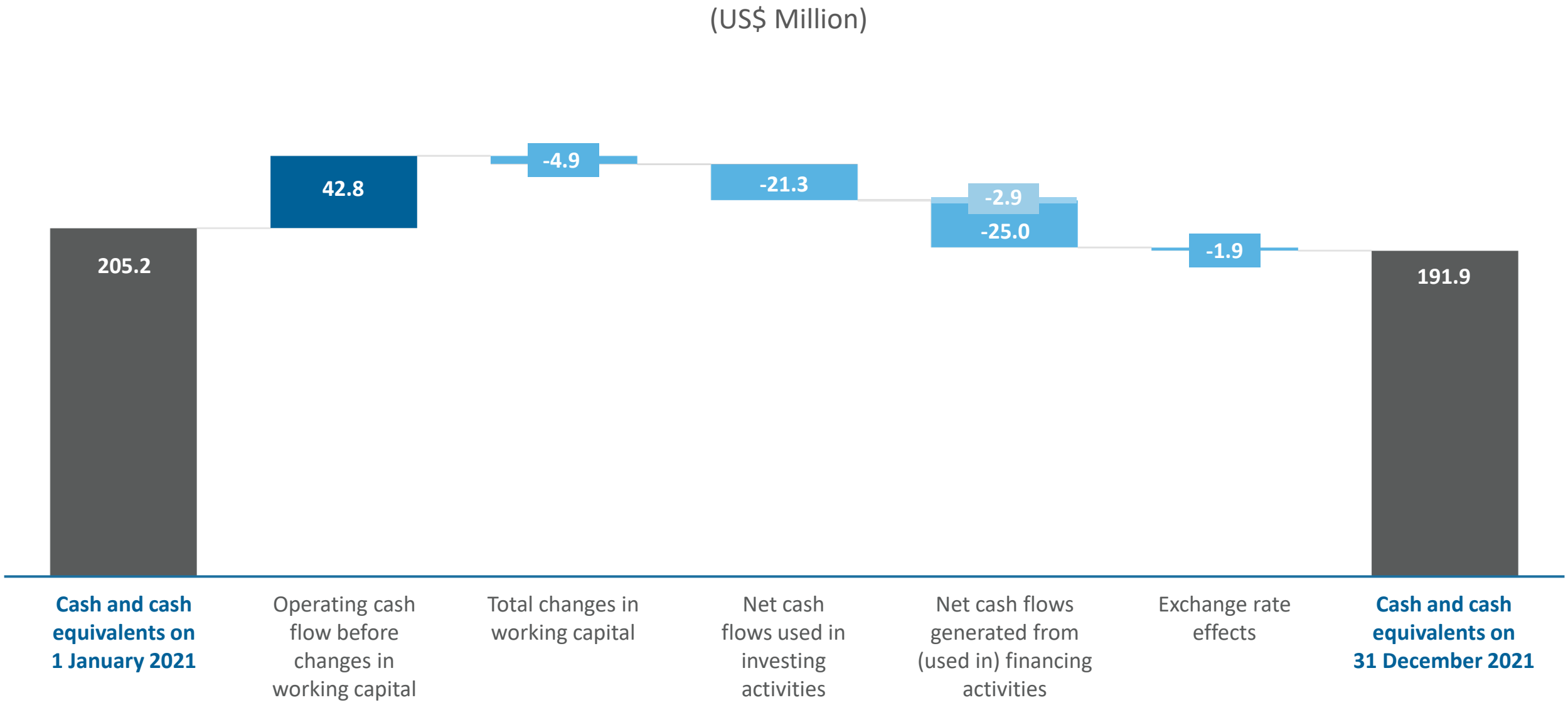
FX effect

Orbimed loan settlement expense 2020

Other

Profit before tax 2021

2021: Cashflow 1 January 2021 – 31 December 2021 (6/6)



Outlook

- A return to single digit growth in Group revenues from RUCONEST® sales, driven by the US and expanded EU operations, subject to the progression of the COVID-19 pandemic. Quarterly fluctuations in revenues are expected.
- The submission of leniolisib regulatory filings to FDA and EMA, with commercial launch expected from early Q1 2023 onwards, subject to regulatory approvals.
- The company will invest in this new product opportunity to accelerate future growth. Investments in launch preparations and focused clinical development for leniolisib will significantly increase and will significantly impact profit. With continued cash flow from RUCONEST® to fund these investments, no additional financing to support the current business is expected.
- Focused investment in potential acquisitions and in-licensing of new late-stage development opportunities and assets in rare and ultra-rare diseases. Financing, if required, would come via a combination of our strong balance sheet and access to capital markets.
- Continued focus on our strategic development, ensuring Pharming's growth through developed assets and a potentially expanded pipeline of in-licensed products to provide further life-saving therapies for patients with unmet medical needs and increase returns for our shareholders.

Q&A

Anurag Relan
Chief Medical Officer

Sijmen de Vries
Chief Executive Officer

Jeroen Wakkerman
Chief Financial Officer

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Euronext Amsterdam: PHARM

NASDAQ: PHAR

Bloomberg: PHAR.AS

This presentation and a recording of this call will be made available on the company's website.

Statement of profit and loss

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Amounts in US$ '000	2021	2020
Revenues	**198,871**	**212,174**
Costs of sales	**(21,142)**	**(23,539)**
Gross profit	**177,729**	**188,635**
Other income	**2,620**	**1,829**
Research and development	(70,369)	(38,519)
General and administrative	(36,974)	(24,085)
Marketing and sales	(59,445)	(51,604)
Other Operating Costs	**(166,788)**	**(114,208)**
Operating profit	**13,561**	**76,256**
Fair value gain (loss) on revaluation derivatives	114	69
Other finance income	14,906	715
Other finance expenses	(6,196)	(33,308)
Finance result, net	**8,824**	**(32,524)**
Share of net profits in associates using the equity method	**694**	**362**
Profit before tax	**23,079**	**44,094**
Income tax expense	(7,082)	(6,348)
Profit for the year	**15,997**	**37,746**
Basic earnings per share (US$)	0.025	0.058
Diluted earnings per share (US$)	0.023	0.055

Balance sheet – assets

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Amounts in US$ '000	2021	2020
Non-current assets		
Intangible assets	83.834	94.083
Property, plant and equipment	13.222	12.226
Right-of-use assets	19.943	9.427
Long-term prepayments	194	0
Deferred tax assets	21.216	31.877
Investment accounted for using the equity method	7.201	7.118
Investments in equity instruments designated as at FVTOCI	1.449	0
Restricted cash	812	510
Total non-current assets	**147.871**	**155.241**
Current assets		
Inventories	27.310	21.157
Trade and other receivables	29.983	35.901
Restricted cash	227	995
Cash and cash equivalents	191.924	205.159
Total current assets	**249.444**	**263.212**
Total assets	**397.315**	**418.453**

Balance sheet – liabilities

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Equity		
Share capital	7.282	7.165
Share premium	453.190	445.066
Legal reserves	2.172	19.859
Accumulated deficit	(269.727)	(288.655)
Shareholders' equity	**192.917**	**183.435**
Non-current liabilities		
Convertible bonds	139.007	149.727
Lease liabilities	18.456	8.230
Other financial liabilities	165	212
Total non-current liabilities	**157.628**	**158.169**
Current liabilities		
Convertible bonds	1.879	2.040
Derivative financial liabilities	0	181
Trade and other payables	42.472	47.666
Lease liabilities	2.419	1.962
Other financial liabilities	0	25.000
Total current liabilities	**46.770**	**76.849**
Total equity and liabilities	**397.315**	**418.453**

Cash flow (1/2)

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Amounts in $'000	2021	2020
Profit before tax	**23.079**	**44.094**
Non-cash adjustments:		
Depreciation, amortization, impairment of non-current assets	19.610	8.314
Equity settled share based payments	9.056	6.537
Fair value gain (loss) loss on revaluation of derivatives	(114)	(69)
Other finance income	(14.906)	(713)
Other finance expenses	6.196	33.308
Share of net profits in associates using the equity method	(694)	(362)
Other	524	(1.624)
Operating cash flows before changes in working capital	**42.751**	**89.485**
Changes in working capital:		
Inventories	(6.153)	(4.934)
Trade and other receivables	5.918	(7.040)
Payables and other current liabilities	(5.193)	7.019
Restricted cash	467	1.039
Total changes in working capital	(4.961)	(3.916)
Interest received	53	715
Income taxes paid	0	(2.658)
Net cash flows generated from (used in) operating activities	**37.843**	**83.626**

Cash flow (2/2)

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Capital expenditure for property, plant and equipment	(10.739)	(4.657)
Investment intangible assets	(3.447)	(9.060)
Investment associate	0	(329)
Investment in equity instruments designated as at FVTOCI	(4.589)	0
Acquisition of license	(2.530)	(1.583)
Net cash flows used in investing activities	**(21.305)**	**(15.629)**
Repayment on loans and borrowings	0	(57.231)
Payment on contingent consideration	(25.000)	(20.722)
Payment of lease liabilities	(3.217)	(2.186)
Proceeds of issued convertible bond	0	142.825
Transaction costs related to issued convertible bond	0	(2.649)
Interests on loans	(4.448)	(2.142)
Proceeds of equity and warrants	4.718	2.791
Net cash flows generated from (used in) financing activities	**(27.947)**	**60.686**
Increase (decrease) of cash	**(11.409)**	**128.683**
Exchange rate effects	(1.826)	2.128
Cash and cash equivalents at 1 January	205.159	74.348
Total cash and cash equivalents at December 31	**191.924**	**205.159**

PI3KCD Patients (N=6): Clinical Demographics

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Patient ID	Age at enrollment (years)	Splenomegaly Lymph-adenopathy	Cytopenia	Pulmonary Problems	History of lymphoma
1	17.3	Yes	No	Bronchiectasis, asthma	No
2	24.3	Yes	Thrombocytopenia, Neutropenia	No	Yes, Large B-cell Lymphoma
3	17.3	Yes	No	Asthma, recurrent bronchitis	No
4	20.9	Yes	Lymphopenia, Neutropenia, Anemia	Chronic Sinusitis Airway disease, Bronchiectasis	No
5	25.5	Yes	No	Recurrent infections, bronchiectasis, COPD	Yes, Hodgkin Lymphoma
6	32.4	Yes	Thrombocytopenia	Chronic sinusitis, bronchial wall thickening	Yes, non-Hodgkin Lymphoma

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

Rao VK, et al. Blood. 2017;130(21):2307–2316.

The HSC gene therapy approach

Pharming

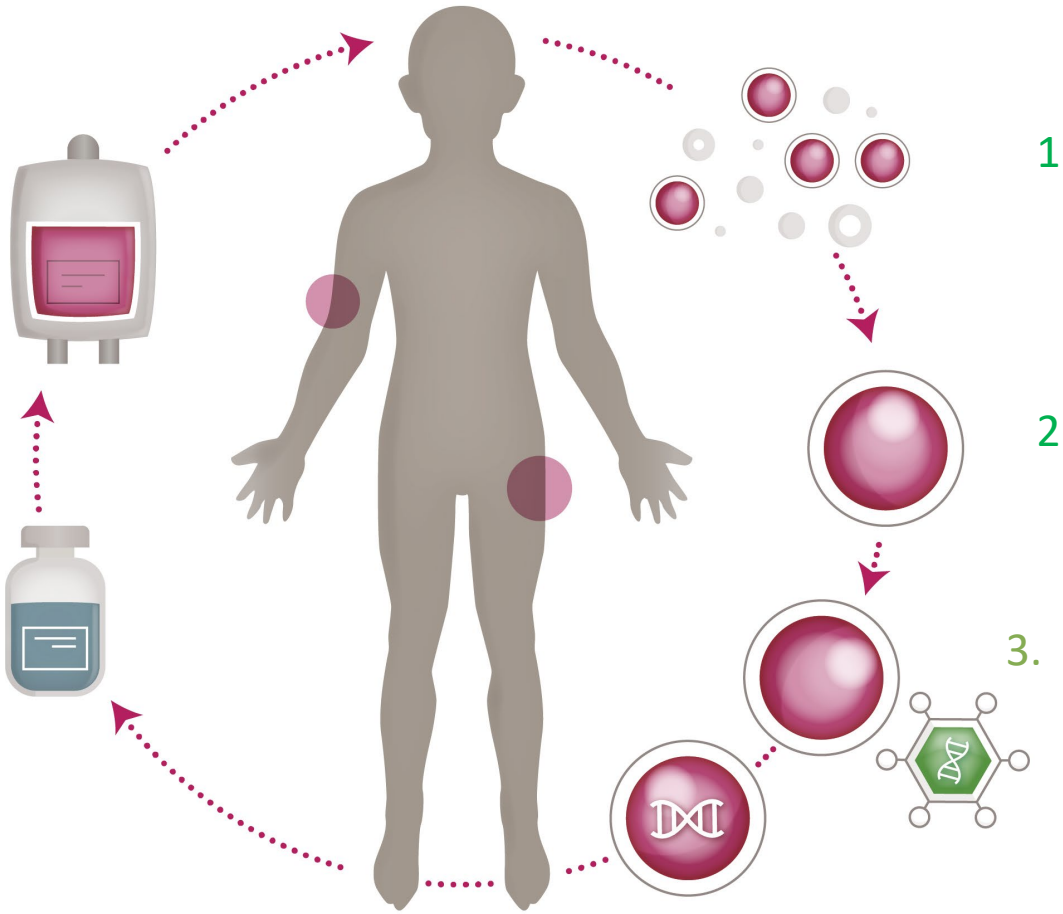
1. Cells are collected from the patient

2. Blood stem cells are selected and purified

3. A working copy of the gene is inserted into the cells using a viral vector
   Gene-corrected cells are frozen, or cryopreserved

4. The patient is conditioned to receive treatment

5. The gene-corrected cells are infused into the patient



HSC gene therapy has led to multiple approved and effective products

Modality	HSC Gene Therapy	AAV- GT	Gene Editing
Proven Approach	✓ – Multiple products approved and pipeline with impressive data – HSC GT and CAR-T drive further innovation	✗ – No liver-directed AAV is approved – Selectivity for specific cells has proven difficult	✗ – No approved products
Efficacy	✓ – Based on other clinical programs, expression levels appear achievable	? – High amount of protein has proven to be very challenging for AAV – Antibodies to AAV	? – Unsure, pre-clinical data appears promising – Rationale based on lanadelumab
Durability of Effect	✓ – Durability of effect has been proven in other programs	? – Decreased expression levels observed Hemophilia A	✓ – Theoretically, should be permanent
Safety	✓ – Autologous HSCT is approved and appears safe	? – Immune responses to target cells – Significant questions remain	? – Promising but no conclusions can be made – No off-switch on kallikrein inhibition