Pharming Group NV

Jefferies Healthcare Conference
New York City

09 June 2022

Forward Looking Statements

This presentation may contain forward-looking statements. Forward-looking statements are neither historical facts nor assurances of future performance. Instead, they are based on our current beliefs, expectations and assumptions regarding the future of our business, future plans and strategies, our development plans, our clinical results and other future conditions. All statements other than statements of historical facts contained in this presentation, including statements regarding our future financial or business performance, conditions, plans, prospects, trends or strategies, objectives of management and other financial and business matters; our current and prospective product candidates, planned clinical trials and preclinical studies, projected research and development costs, current and prospective collaborations; and the estimated size of the market for our product candidates, the timing and success of our development and commercialization of our product candidates and the market acceptance thereof, are forward-looking statements. New risks and uncertainties may emerge from time to time, and it is not possible to predict all risks and uncertainties. While we may elect to update these forward-looking statements at some point in the future, we assume no obligation to update or revise any forward-looking statements except to the extent required by applicable law. Although we believe the expectations reflected in such forward-looking statements are reasonable, we can give no assurance that such expectations will prove to be correct. Accordingly, readers are cautioned not to place undue reliance on these forward-looking statements. No representations or warranties (expressed or implied) are made about the accuracy of any such forward-looking statements.

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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 expected to continue to grow single digits in 2022 as result of increasing patient demand in the treatment of HAE
- Near-term inflection point with anticipated launch of leniolisib from Q1 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 400 patients already identified
- 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
- 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

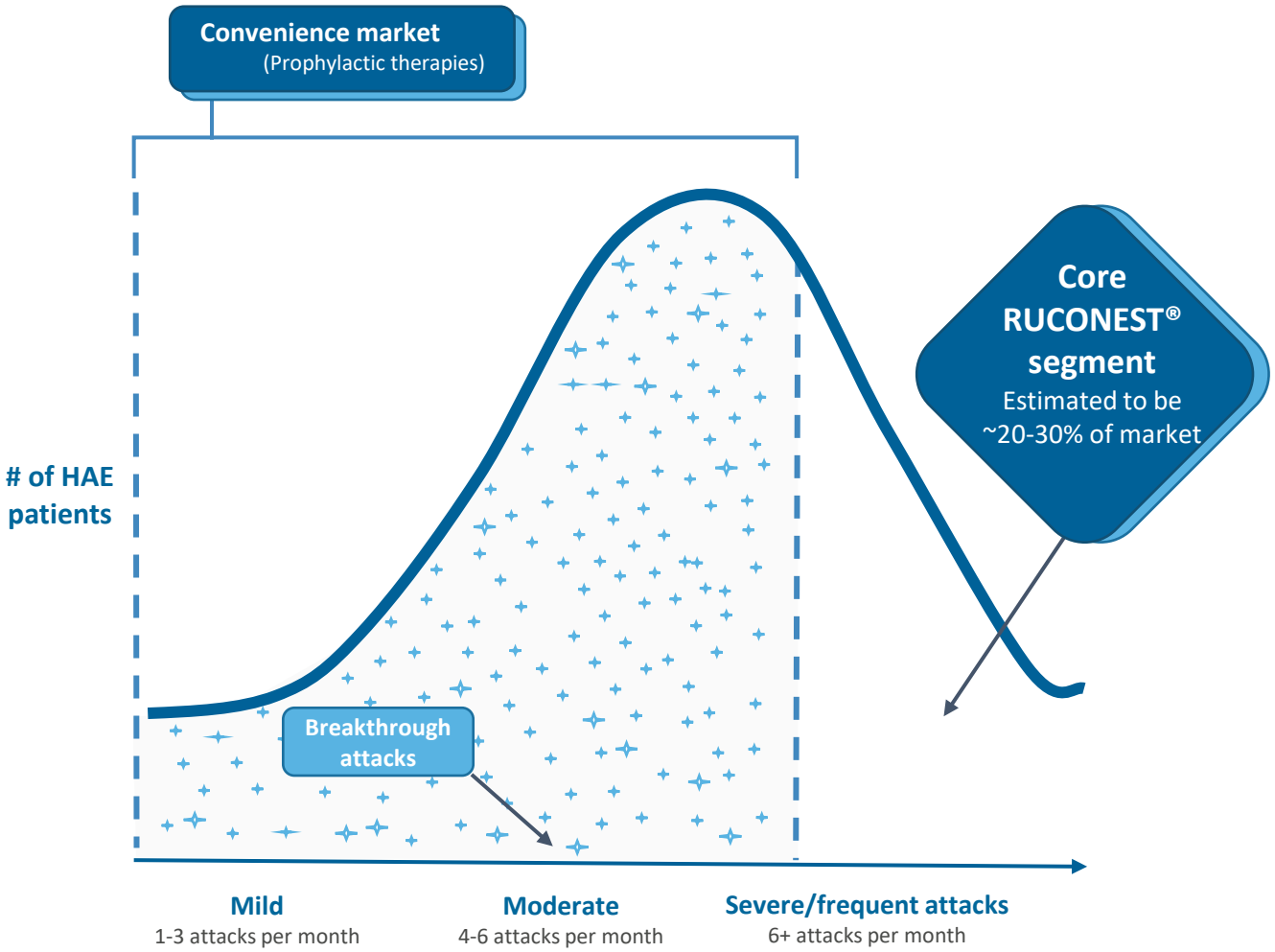
- Development of early stage OTL-105, an ex-vivo HSC gene therapy candidate for HAE
- Development of early-stage asset, rhaGLU, an enzyme replacement therapy for Pompe disease

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

APDS & leniolisib

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

PI3Kδ hyperactivity leads to APDS symptoms

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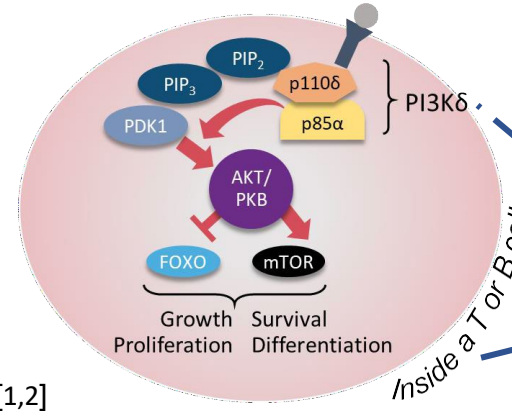
Hyperactive PI3Kδ activity



[1,2]

Inside a T or B cell

Excess of immature or senescent cells

Deficit of functional cells

Dysregulated T & B cell development

↑ CD8+ effector/memory T cells
↑ CD8+ T cell senescence
↻ Inverted CD4+/CD8+ T cell ratio
↑ Transitional B cells
↔ or ↑ IgM

↓ Naïve and CD4+ T cells
↓ Memory T cell function
↓ B cells (lymphopenia)
↓ Memory B cells
↔ or ↓ IgG/IgA

[3-6]

Common Symptoms of APDS[4,5]

Severe, Recurrent, Persistent Infections:
- Sinopulmonary
- Herpesvirus (especially EBV and CMV)

Lymphoproliferation:
- Lymphadenopathy
- Splenomegaly/hepatomegaly
- Nodular lymphoid hyperplasia

Autoimmunity:
- Cytopenias
- Autoimmune disorders
- Autoinflammatory disorders

Bronchiectasis

Enteropathy

Sclerosing Cholangitis & Cirrhosis

Lymphoma and Other Cancers

Developmental Delay and other cognitive symptoms may be due to PI3Kδ expression in other cell types such as neurons

APDS, activated PI3Kδ syndrome; CD, cluster of differentiation; CMV, cytomegalovirus; EBV, Epstein-Barr virus; FOXO, forkhead box O; Ig, immunoglobulin; mTOR, mammalian target of rapamycin; PDK1, phosphoinositide-dependent protein kinase 1; PIP_2, phosphatidylinositol 4,5-bisphosphate; PIP_3, phosphatidylinositol 3,4,5-trisphosphate; PI3Kδ, phosphoinositide 3-kinase delta; PKB, protein kinase B.

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.

Patient demographics – safety analysis

	Leniolisib (n=21)	Placebo (n=10)	Total (N=31)
Age Median (range), years < 18 years, n (%)	 20.0 (12-54) 8 (38.1)	 19.5 (15-48) 4 (40.0)	 20.0 (12-54) 12 (38.7)
Sex: Male/female, %	52.4/47.6	40.0/60.0	48.4/51.6
Weight: Median (range), kg	67.1 (46.9-100.6)	68.9 (50.0-88.0)	67.1 (46.9-100.6)
Variant: *PIK3CD/PIK3R1, %*	76.2/23.8	90.0/10.0	80.6/19.4
Baseline glucocorticoids,* %	58.1	60	57.1
Baseline IRT,† %	66.7	70.0	68.7

	Total (N=31), %
Lymphoproliferation	93.5
Chronic infections	90.3
Pulmonary disease Bronchiectasis	64.5 61.3
Cytopenias	61.3
Gastrointestinal disease	54.8

Other notable characteristics:

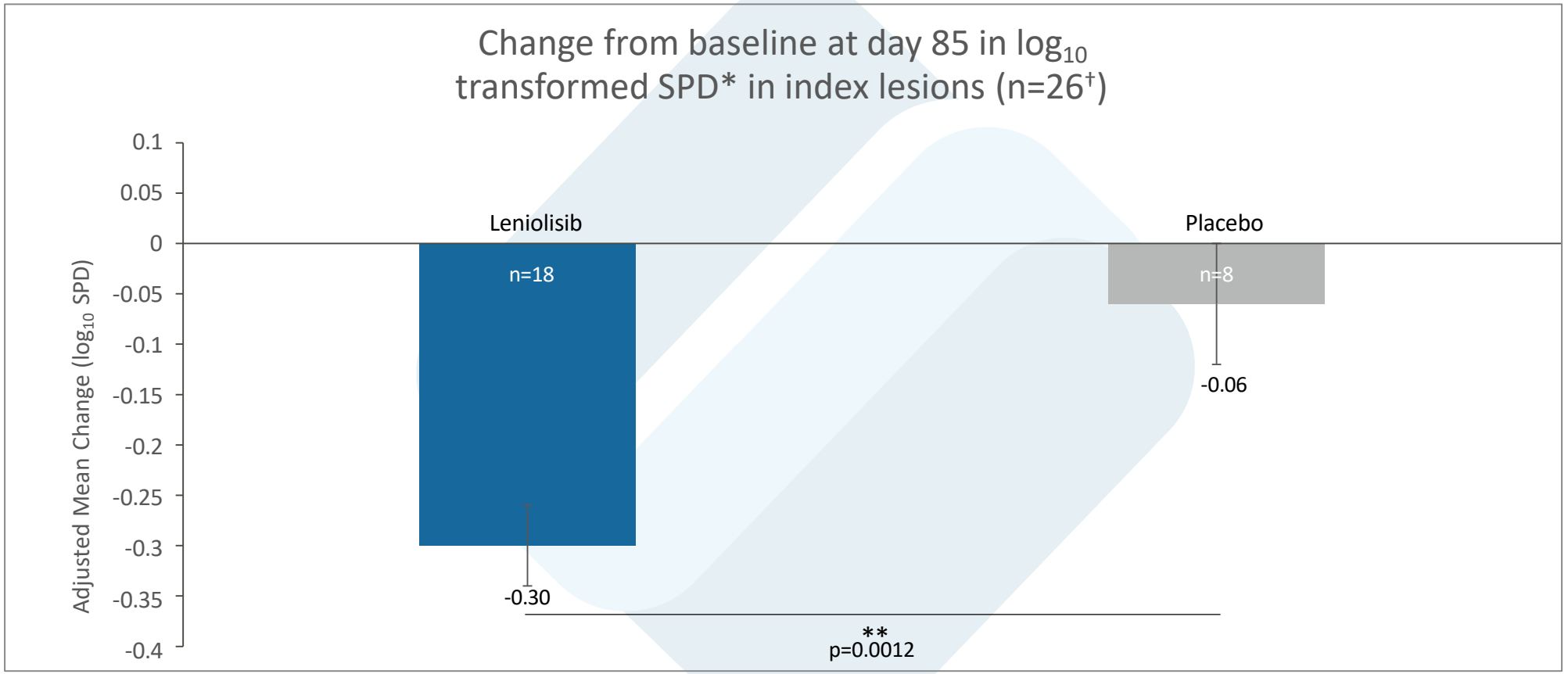
Short stature observed in 2 patients with APDS1 and 4 patients with APDS2

32.3% of patients had neurological manifestations, including 19.4% of patients with anxiety

23% of patients were previously treated with sirolimus**

*Systemic glucocorticoids below 25 mg prednisone or equivalent per day within 2 weeks prior to first dosing of study medication were permitted. †Analyses using baseline IVIG as a categorical (Yes/No) covariate used different data.
**Note that these numbers include additional data collected from investigators that is outside of the clinical study report.

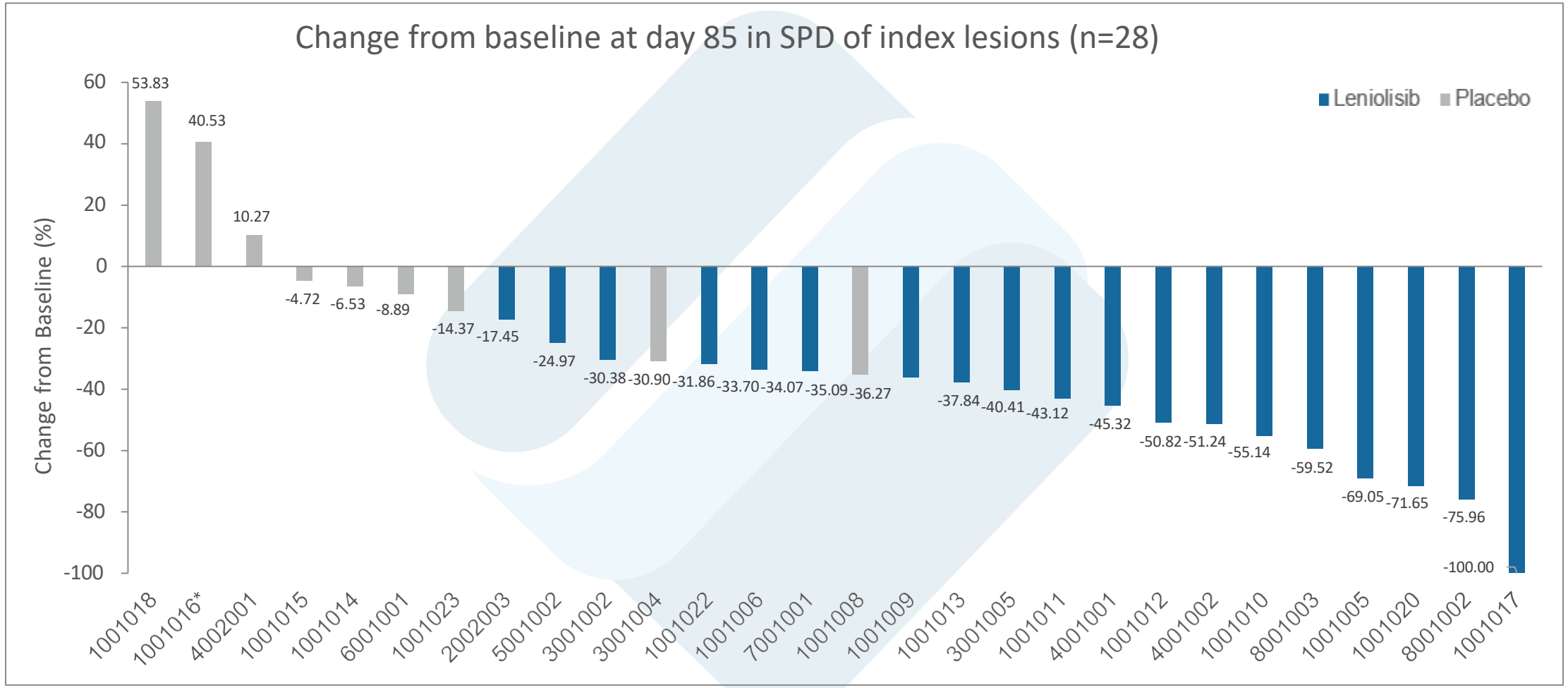
Leniolisib reduced lymphadenopathy



Data were analyzed using ANCOVA model with treatment as a fixed effect and log_{10} transformed baseline SPD as a covariate. Use of glucocorticoids and IVIG at baseline were both included as categorical (Yes/No) covariates. P-value is 2-sided. Error bars are standard error of the mean.

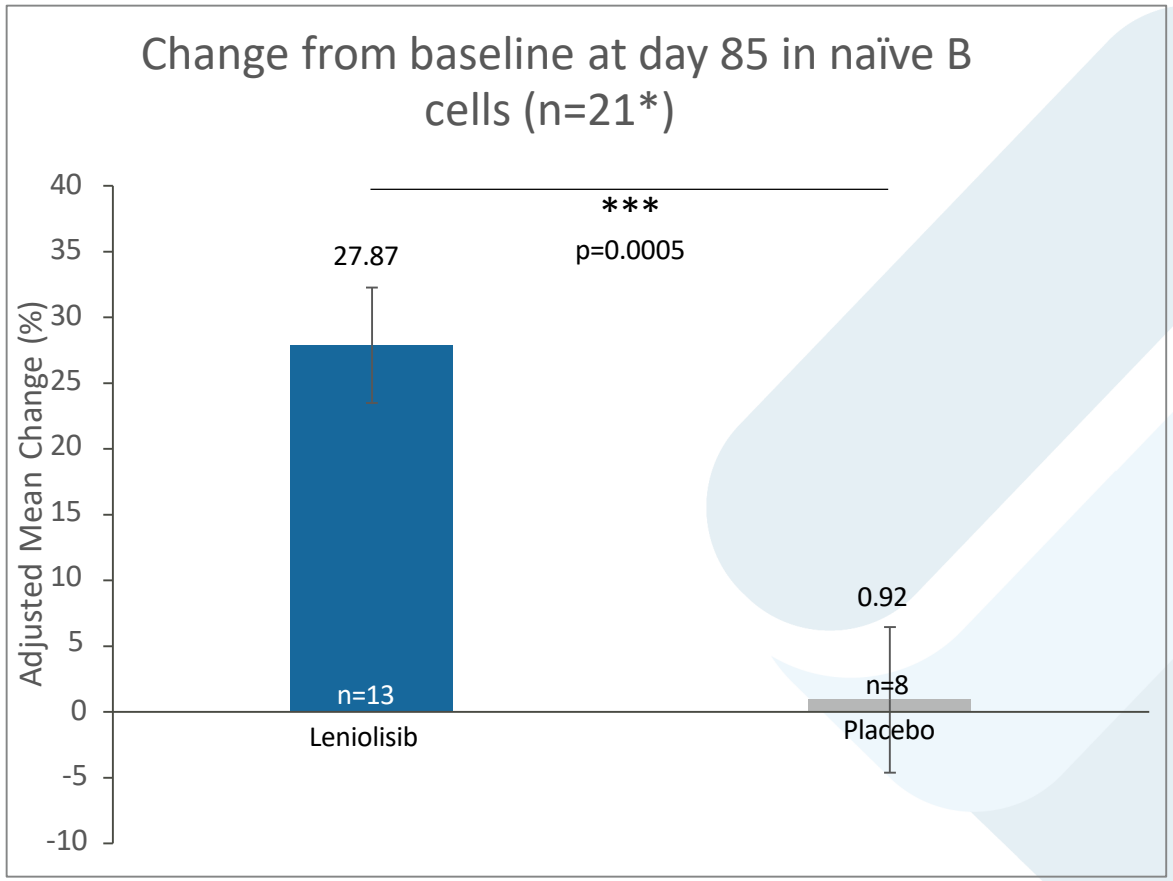
*Longest lesion diameter (mm) and longest perpendicular diameter (mm) for each index lesion were used to calculate the log_{10} transformed SPD. †4 patients from the 31 in the safety analysis were excluded from the PD analysis. An additional patient was excluded from the index lesion analysis because the baseline lung index had fully resolved (0 mm) by day 85.

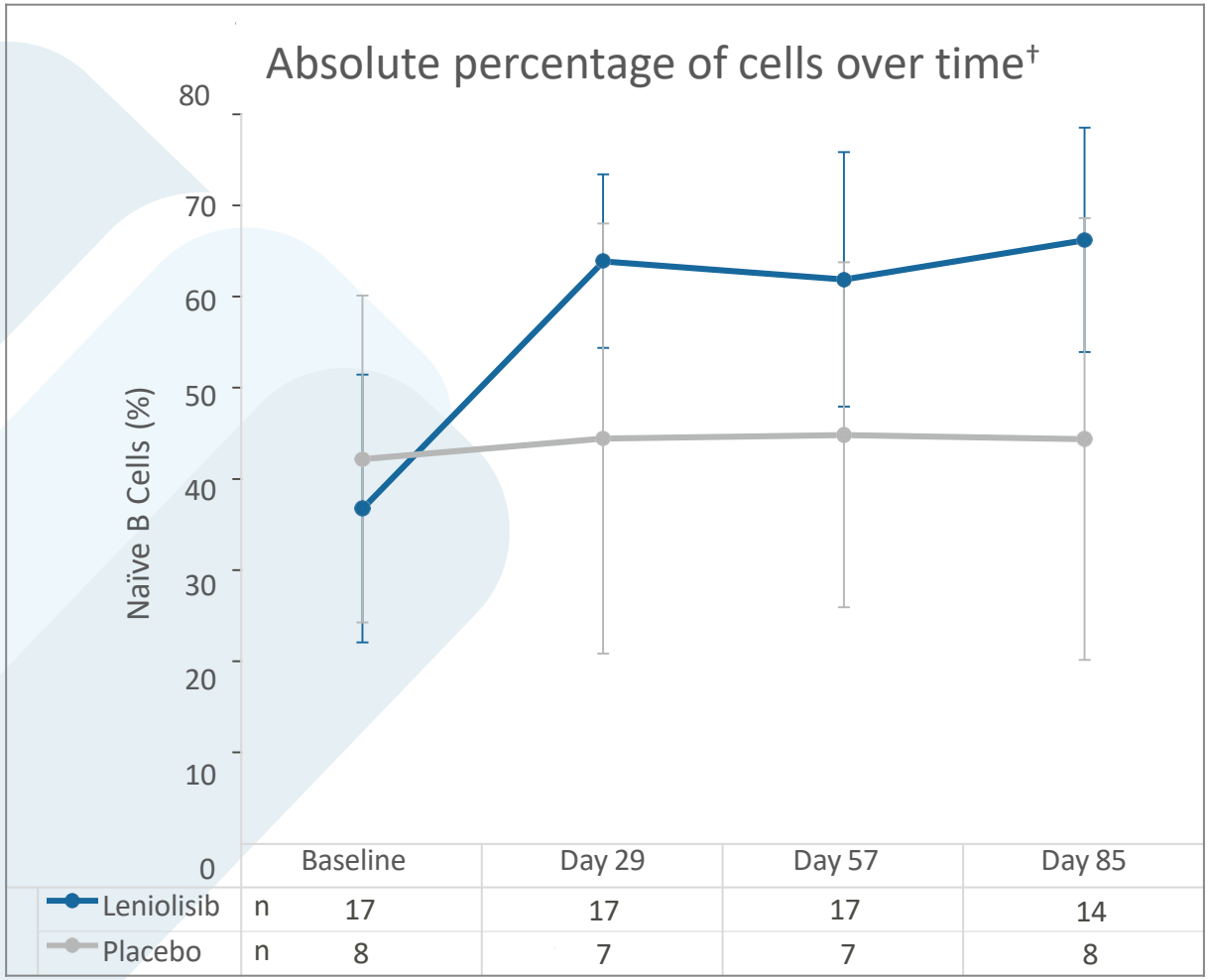
Additional analysis: SPD of index lesions by patient in PD data



*This patient was excluded from the PD analysis due to prednisone use > 25 mg within 14 days of first dose.

Supportive analysis: naïve B cells



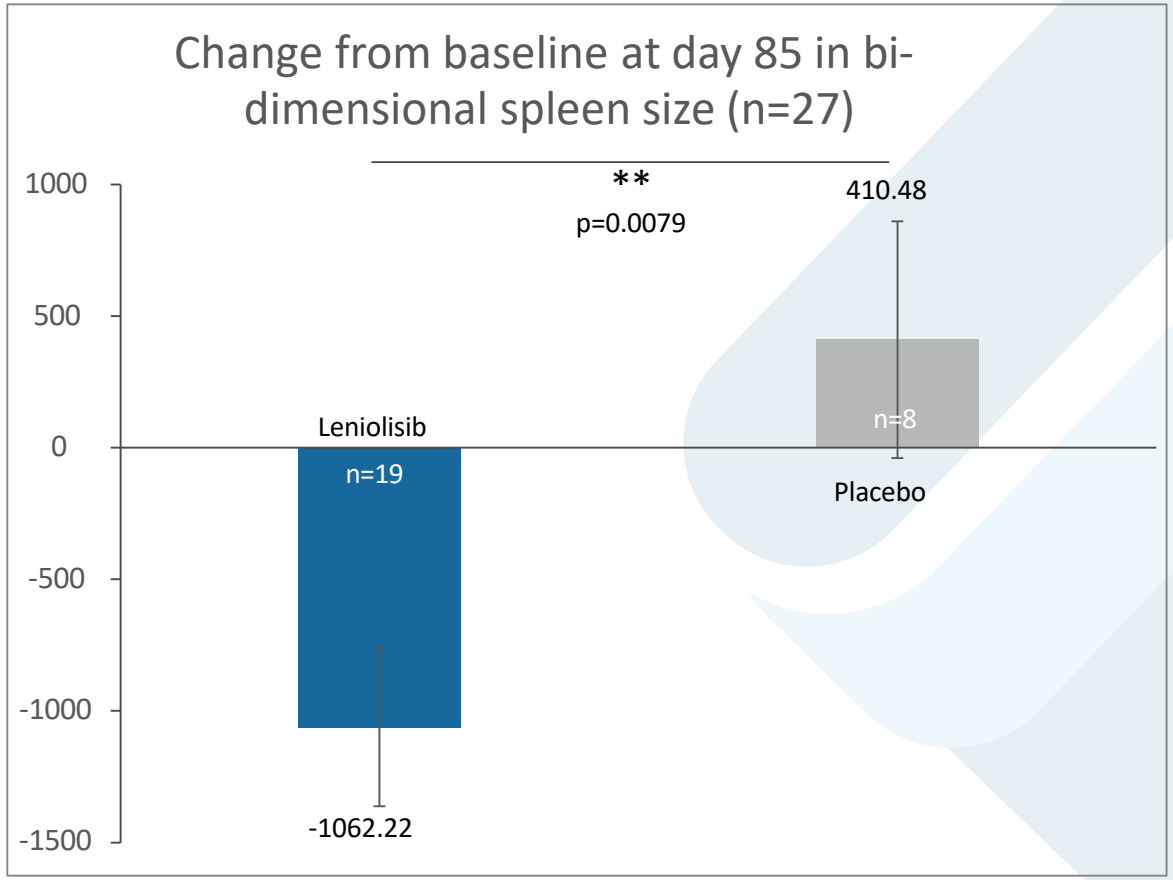


The PD analysis set was used for this supportive analysis. Only subjects with a derived baseline value and a result at that time point are included.

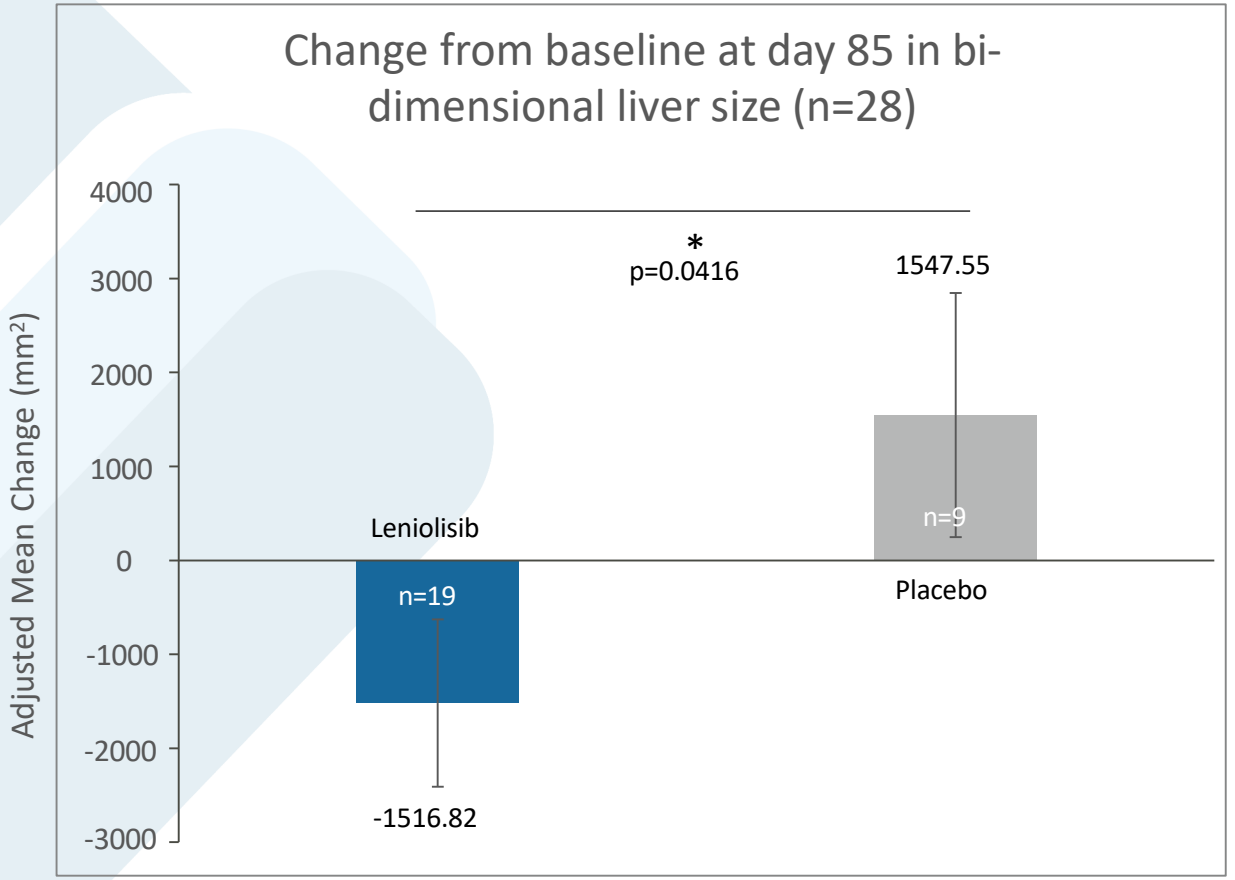
*Data were analyzed using an ANCOVA model with treatment as a fixed effect and baseline as a covariate. Use of glucocorticoids and IVIG at baseline were both included as categorical (Yes/No) covariates. *Baseline* is defined as the arithmetic mean of the baseline and Day 1 values when both are available, and if either baseline or the Day 1 value is missing, the existing value is used. P-value is 2-sided. Error bars are standard error of

Secondary and exploratory analyses: leniolisib reduced spleen and liver size

Spleen



Liver



Data were analyzed using ANCOVA model with treatment as a fixed effect and $\log_{10}$ transformed baseline SPD as a covariate. Use of glucocorticoids and IVIG at baseline were both included as categorical (Yes/No) covariates. P-value is 2-sided. Error bars are standard error of the mean.

Leniolisib over three months was well tolerated

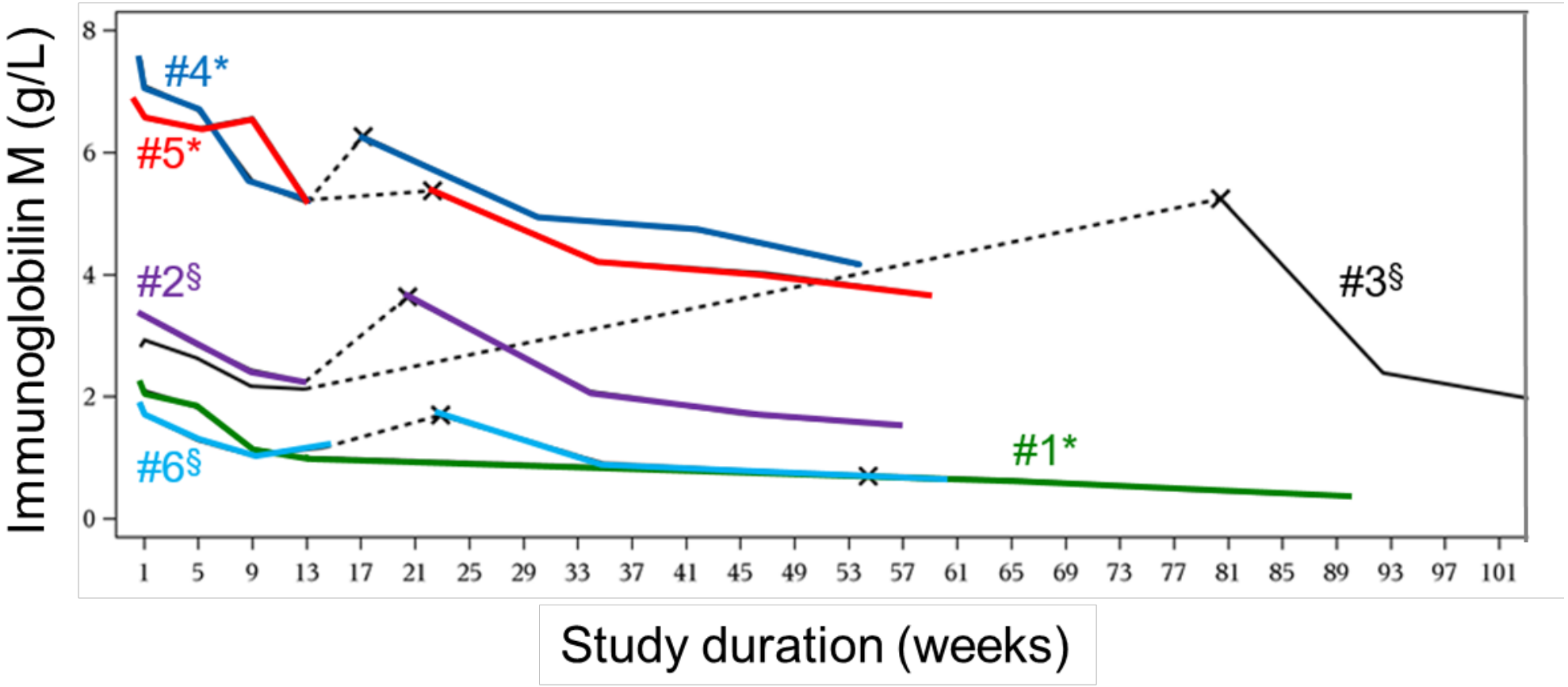
	Leniolisib (n=21) nE, nS (%)*	Placebo (n=10) nE, nS (%)	Total (N=31) nE, nS (%)
AEs, Patients with AEs	92, 18 (85.7)	46, 9 (90.0)	138, 27 (87.1)
Grade 1 AEs	65, 15 (71.4)	27, 8 (80.0)	92, 23 (74.2)
Grade 2 AEs	19, 9 (42.9)	13, 5 (50.0)	32, 14 (45.2)
Grade 3 AEs	3, 2 (9.5)	4, 3 (30.0)	7, 5 (16.1)
Grade 4 AEs	3, 2 (9.5)	1, 1 (10.0)	4, 3 (9.7)
Grade 5 AEs	0	1, 1 (10.0)	1, 1 (3.2)
Study drug-related AEs	6, 5 (23.8)	8,3 (30.0)	14, 8 (25.8)
SAEs	5, 3 (14.3)	6, 2 (20.0)	11, 5 (16.1)

- No deaths were reported
- No AEs led to discontinuation of study treatment

- No SAEs were related to study treatment, and the incidence of SAEs was lower in the leniolisib group than the placebo group

*nE, number of AE events in the category; nS, number of patients with at least 1 AE in the category; % is based on the number of patients.

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.

Launch preparations: Uncovering “APDS”

US targeted patient identification strategy

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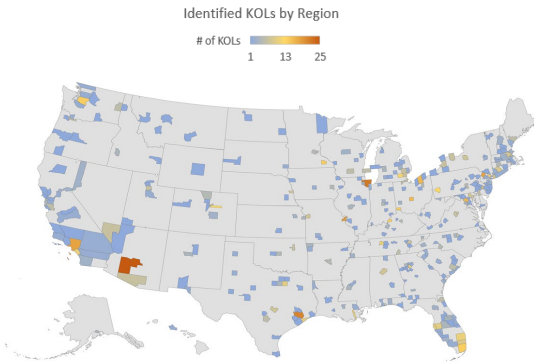
1.

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

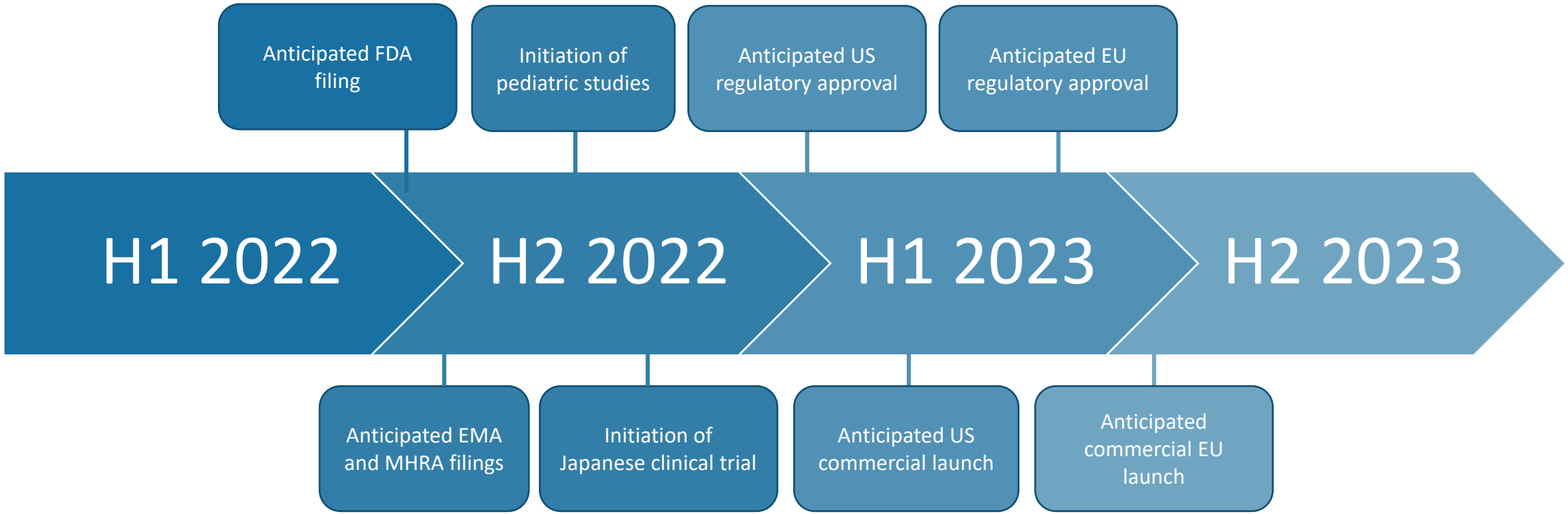
Immune Deficiency Foundation

Global Genes

Jeffrey Modell Foundation

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

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

The HSC gene therapy approach

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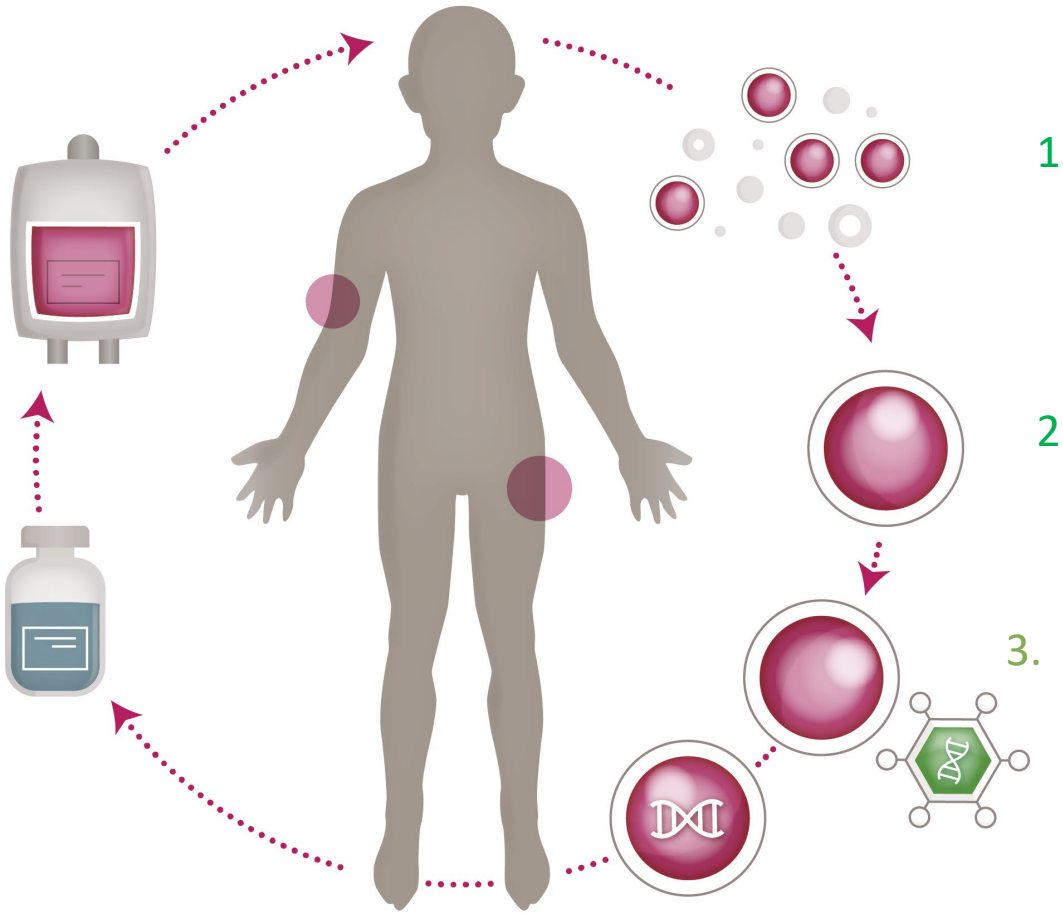
1. Cells are collected from the patient
2. Blood stem cells are selected and purified
3. A working copy of the gene is inserted into the cells using a viral vector
   Gene-corrected cells are frozen, or cryopreserved
4. The patient is conditioned to receive treatment
5. The gene-corrected cells are infused 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

Financial Highlights and Outlook 2022

Investing to expand the business

Financial highlights Q1 2022: Building a sustainable business

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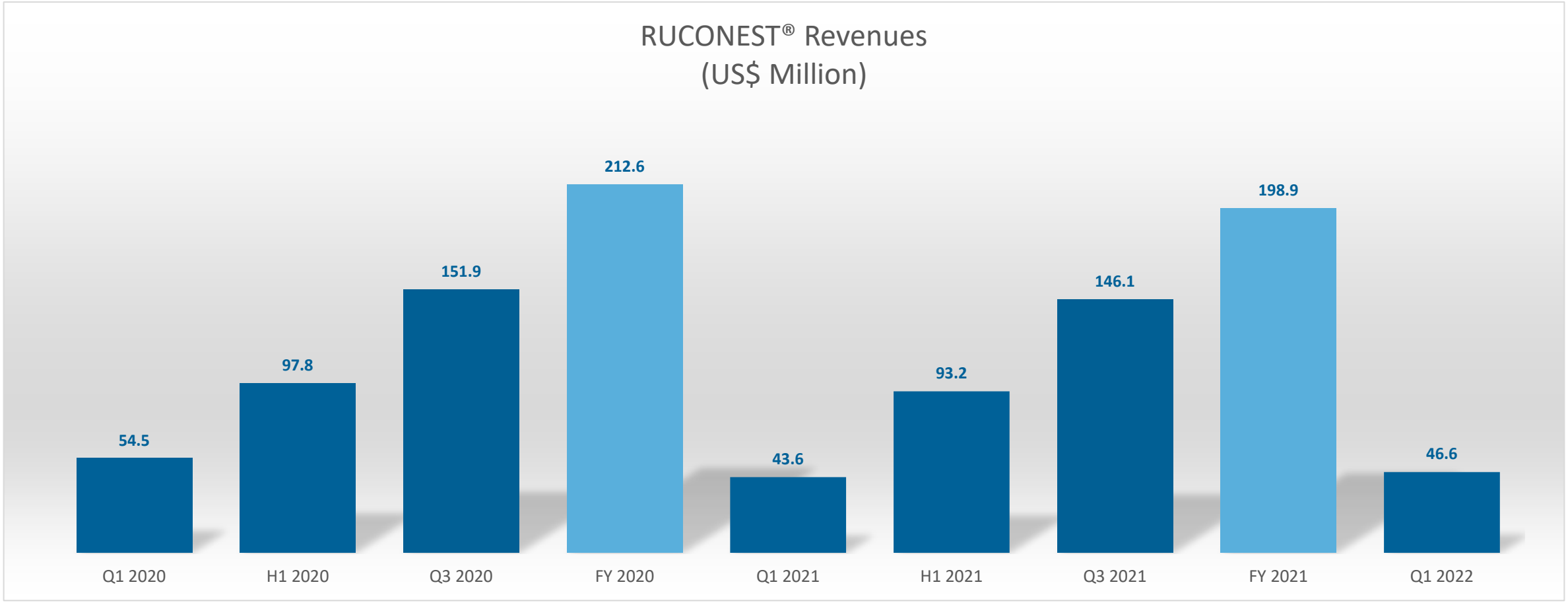
REVENUE

- Total revenues for Q1 2022 increased by 7% to US$46.6 million compared to US$43.6 million in Q1 2021



Financial highlights Q1 2022: Investing to expand the business

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OPERATING PROFIT & COST

- Operating profit decreased to US$2.8 million (Q1 2021: US$6.3m), mainly due to an expected increase in operating expenses from US$32.7 million in Q1 2021 to US$39.8 million in Q1 2022
  - A combination of launch preparations for leniolisib, increased travel expenses post-Covid and phasing of costs

NET PROFIT

- Net profit of US$3.5 million decreased 59% (Q1 2021: US$8.5 million). The decrease was caused as a result of a significant decrease in finance income from US$6.6 million in Q1 2021 to US$1.8 million in Q1 2022, mainly due to more favorable exchange rate gains in Q1 2021
  - Remainder of the decrease relates to increased operating expenses, partly offset by the growth in gross profit

CASH & CASH EQUIVALENTS

- Cash and cash equivalents decreased by US$2.2 million to US$189.7 million from US$191.9 million at the end of Q4 2021
  - Positive cash flows from operations amounted to US$0.6 million in Q1 2022

Outlook for 2022

For the remainder of 2022, the Company expects:

- 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 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.

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