Pharming Group N.V.

Q1 2022 Financial Results
Analyst Call

May 12, 2022

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

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.

This presentation is not a prospectus, and it does not constitute an offer to sell or a solicitation of an offer to buy securities, nor shall there be any sale of any securities in any state or jurisdiction in which such offer, solicitation or sale would be unlawful prior to registration or qualification under the securities laws of any such state or jurisdiction.

Speakers

Sijmen de Vries
Chief Executive Officer

Jeroen Wakkerman
Chief Financial Officer

Anurag Relan
Chief Medical Officer

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 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 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

Pharming

Grow our global fully integrated commercial infrastructure

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

- 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 leveraging our in-house expertise in rare disease/unmet need to grow our business

In-licencing/ acquisition of late-stage development opportunities and assets in rare diseases

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

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

Grow our rare patient identification database with genetic testing and development of existing early-stage assets

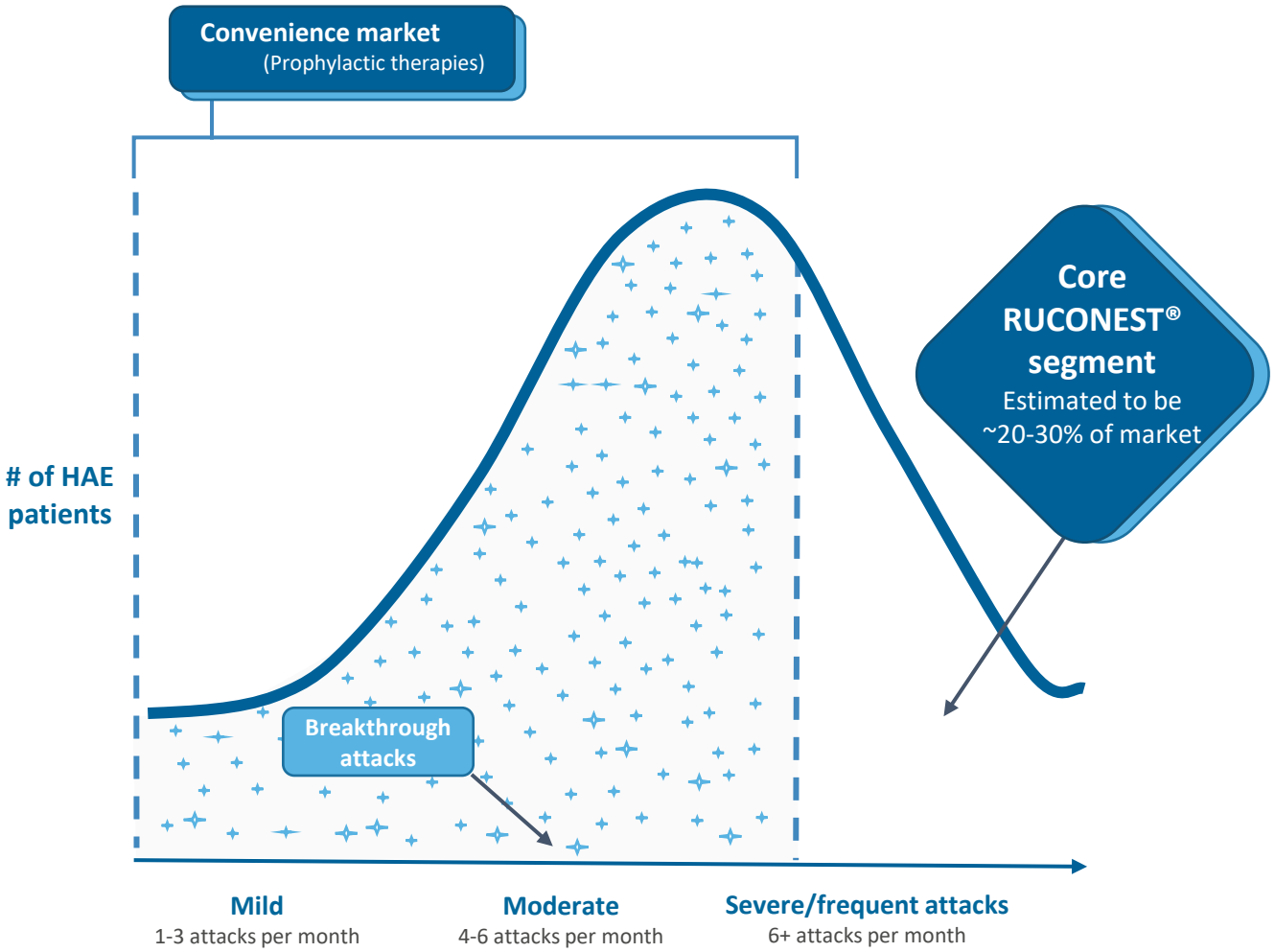
- Development of OTL-105, an ex-vivo HSC gene therapy candidate for HAE
- Development of rhaGLU, an enzyme replacement therapy for Pompe disease

HAE & RUCONEST®

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

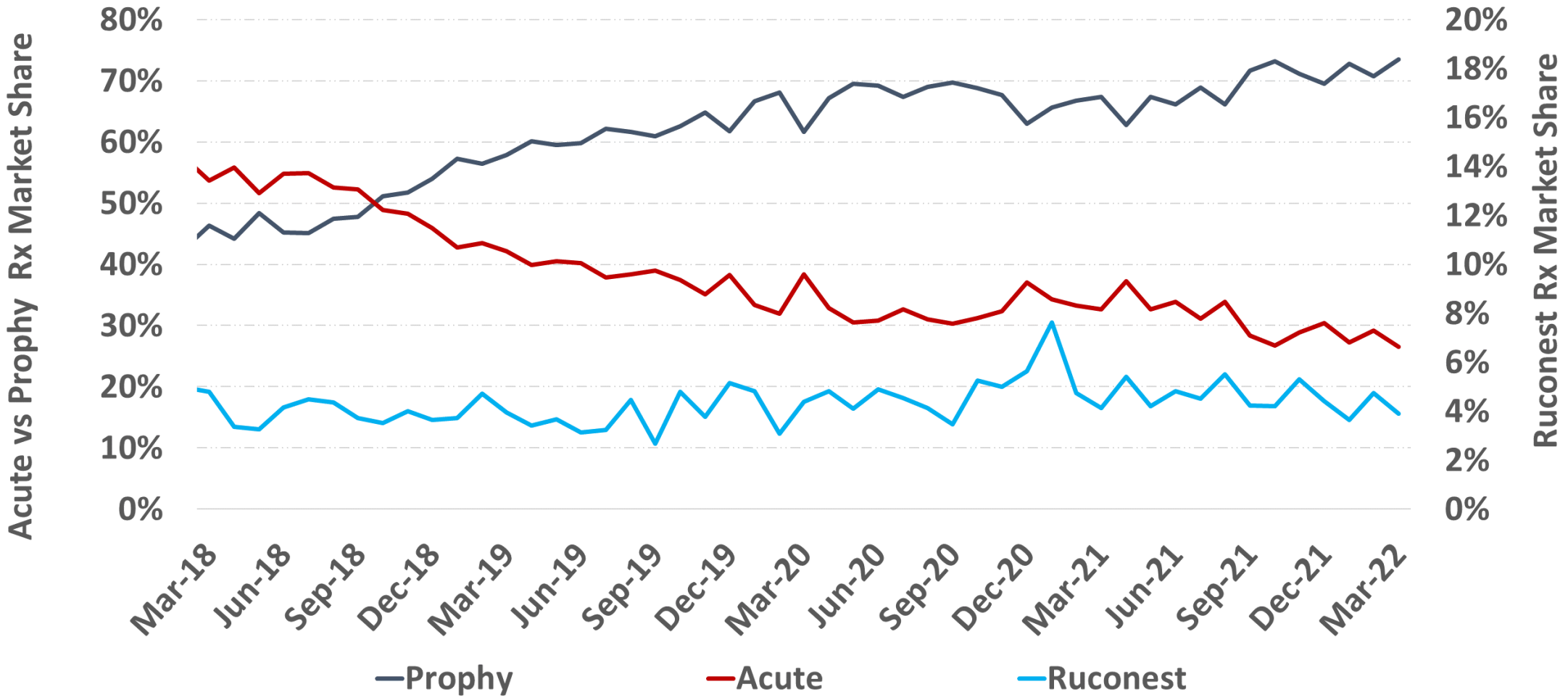
RUCONEST® positioning in the treatment of HAE

- 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

Ongoing demand for acute therapy following stabilization of prophylactic market



Source: *Payer sourced Claims through Mar '22.*

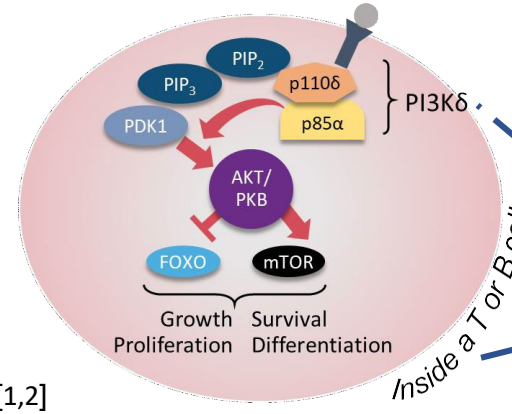
APDS & leniolisib

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

PI3Kδ hyperactivity leads to APDS symptoms

Pharming

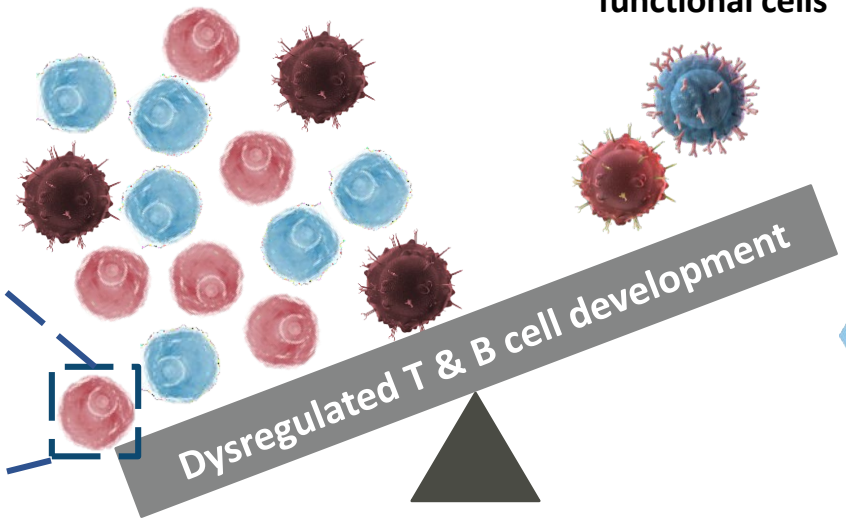
Hyperactive PI3Kδ activity



[1,2]

Excess of immature or senescent cells

Deficit of functional cells



[3-6]

↑ 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

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]

Pharming

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:

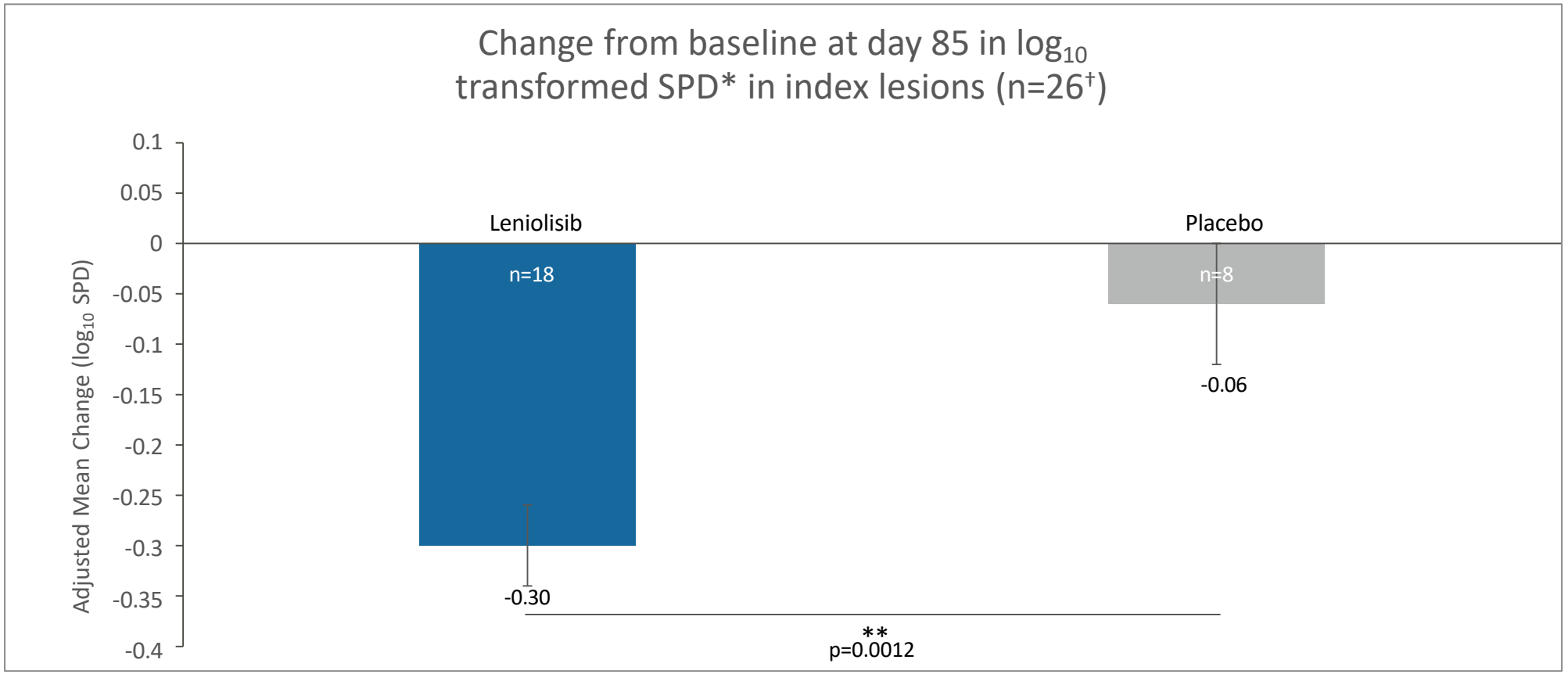
Short stature observed in 2 patients with APDS1 and 4 patients with APDS2

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.

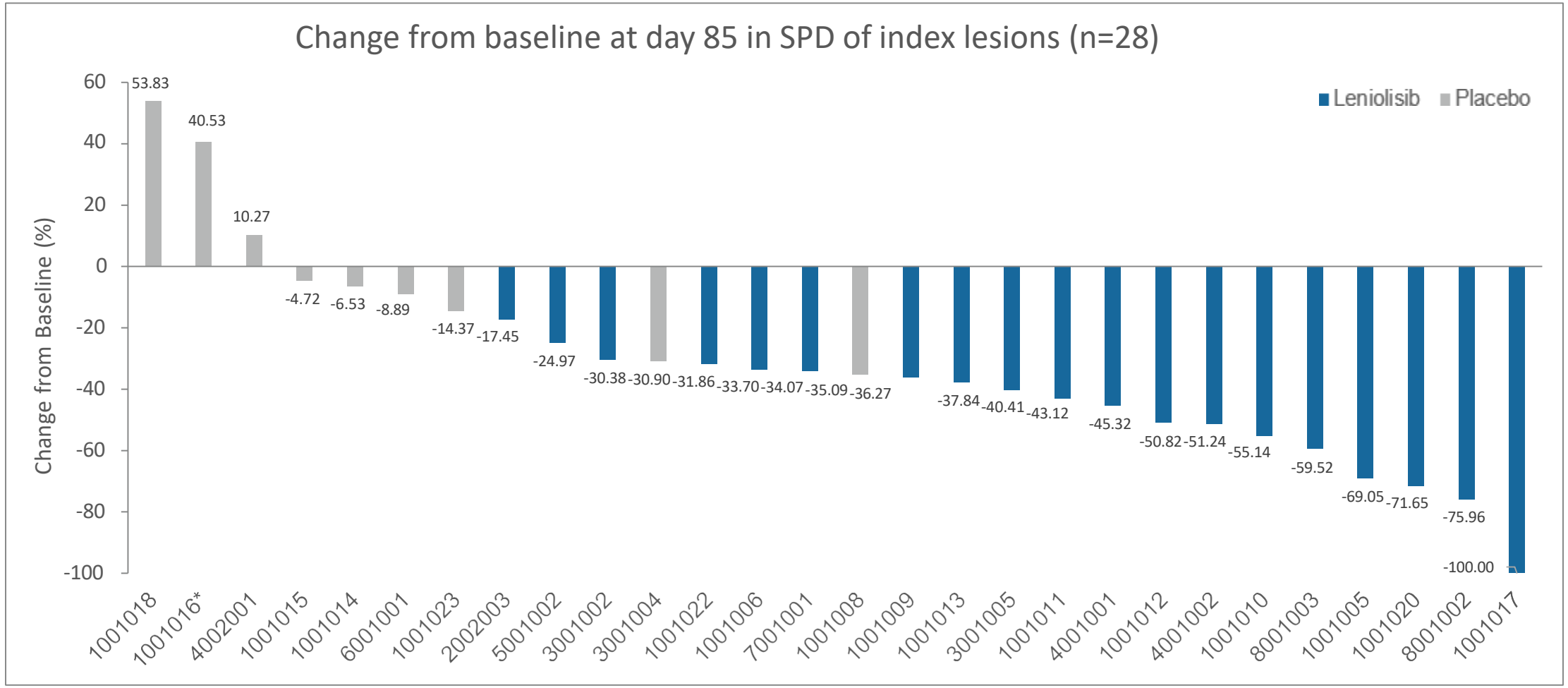
Leniolisib reduced lymphadenopathy



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.

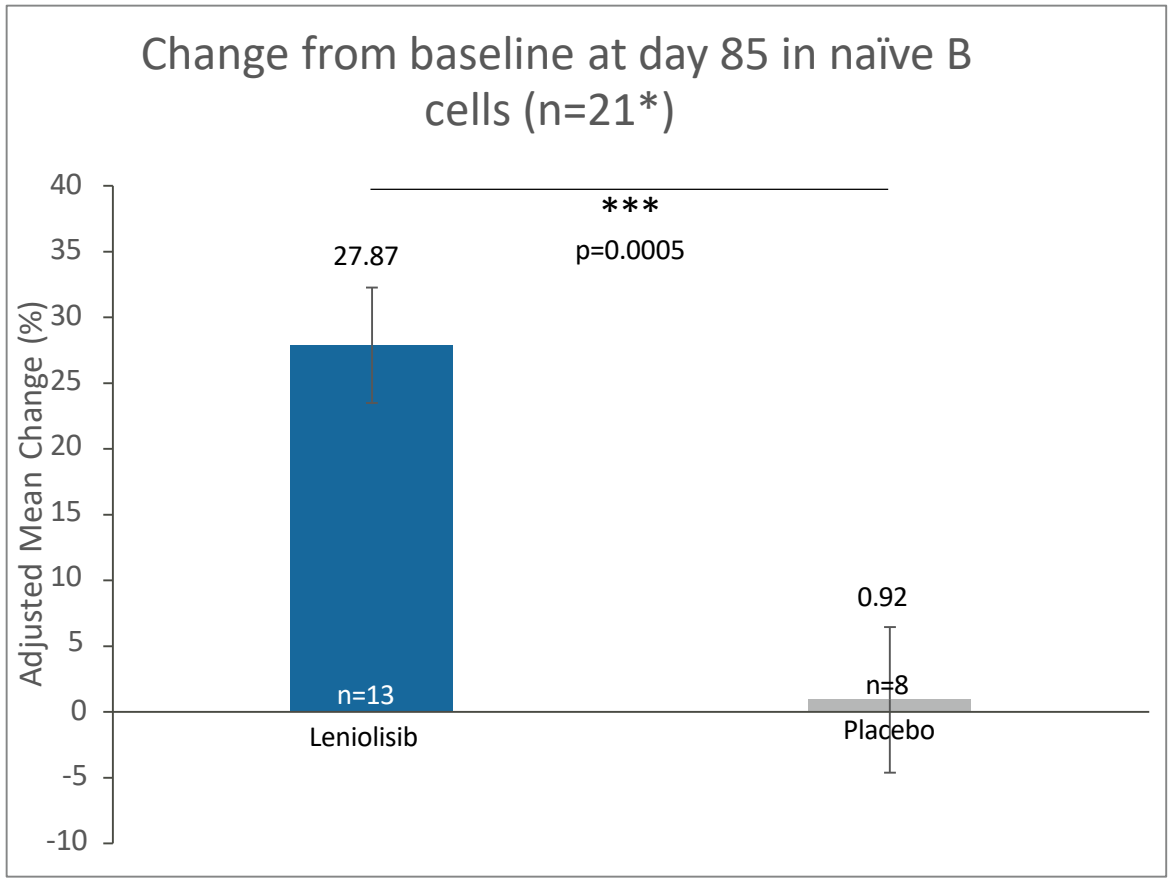
*Longest lesion diameter (mm) and longest perpendicular diameter (mm) for each index lesion were used to calculate the log_{10} transformed SPD. †4 patients from the 31 in the safety analysis were excluded from the PD analysis. An additional patient was excluded from the index lesion analysis because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

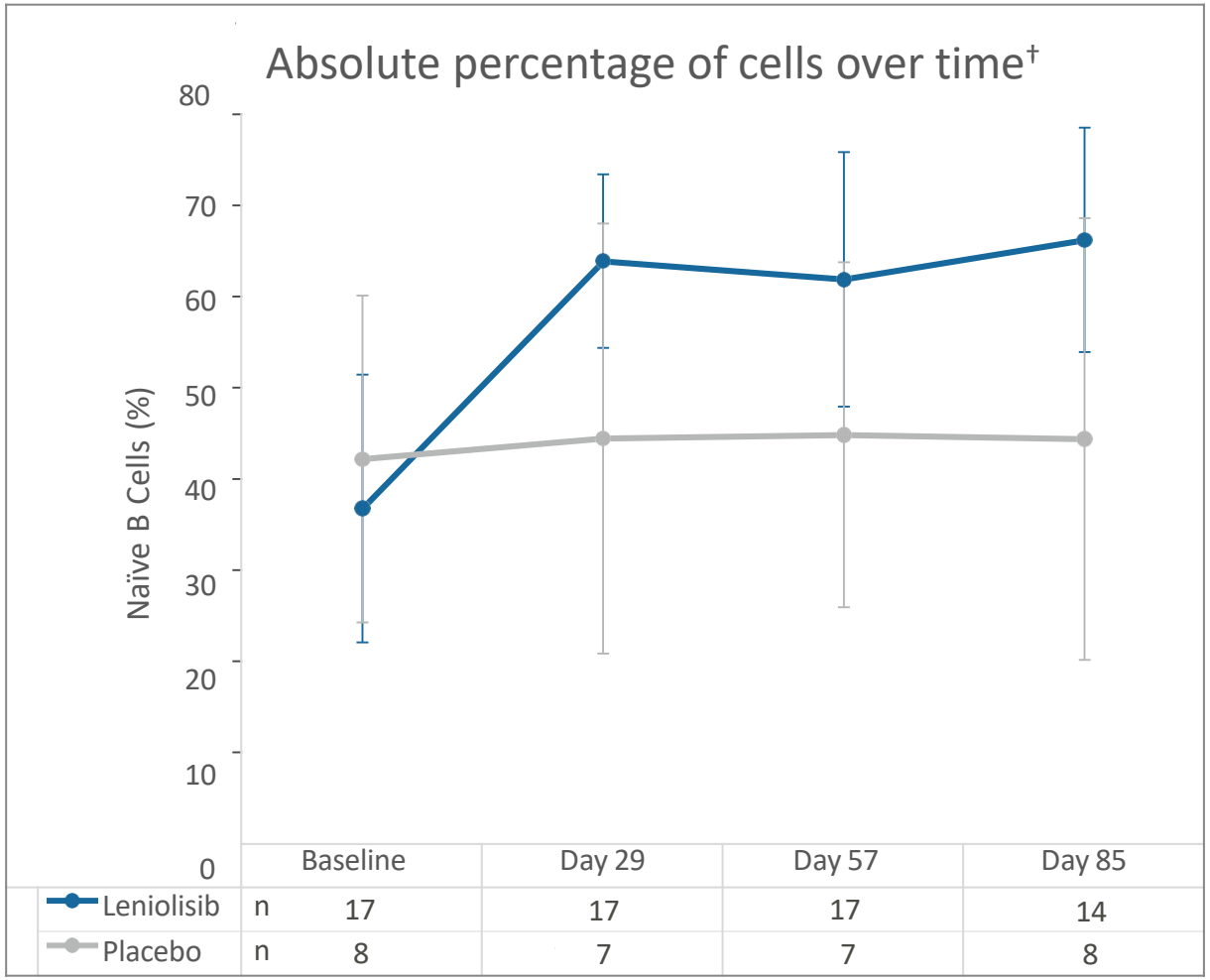
Additional analysis: SPD of index lesions by patient in PD data



*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



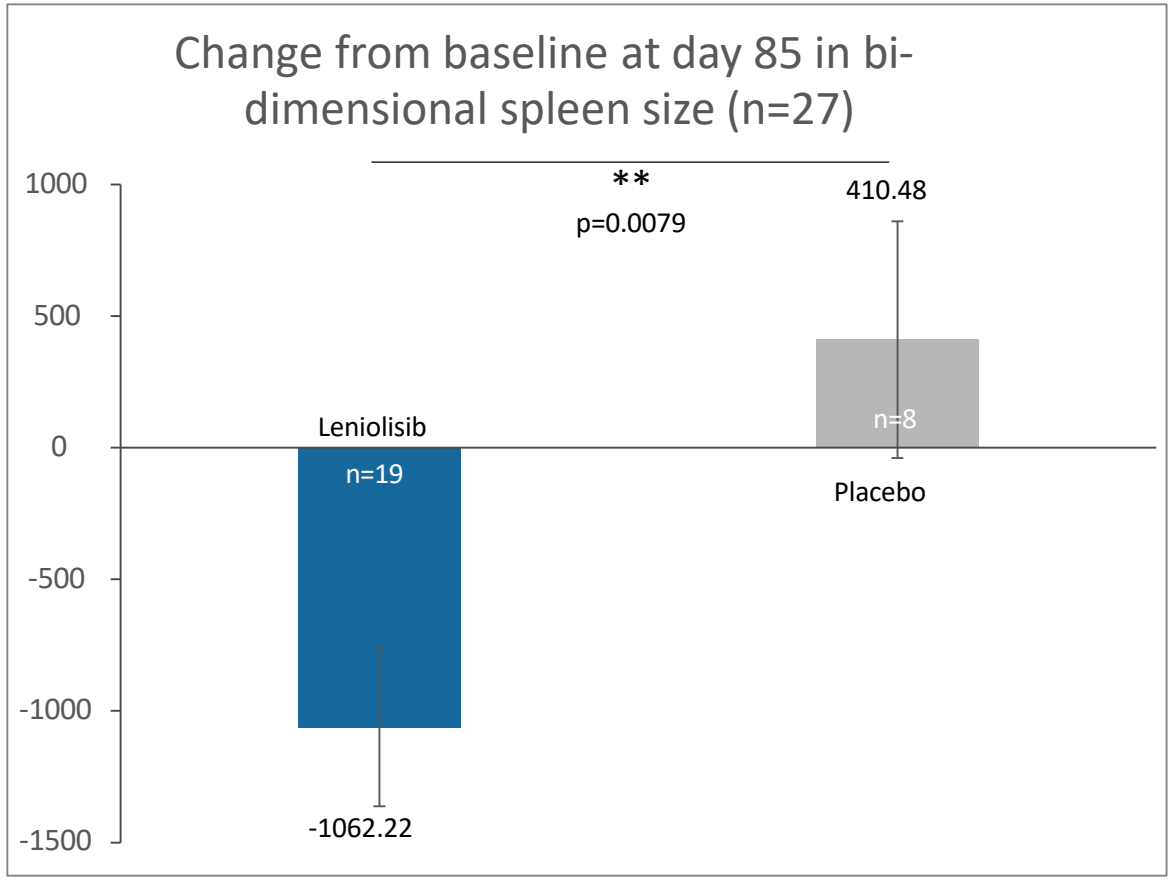


The PD analysis set was used for this supportive analysis. Only subjects with a derived baseline value and a result at that time point are included.

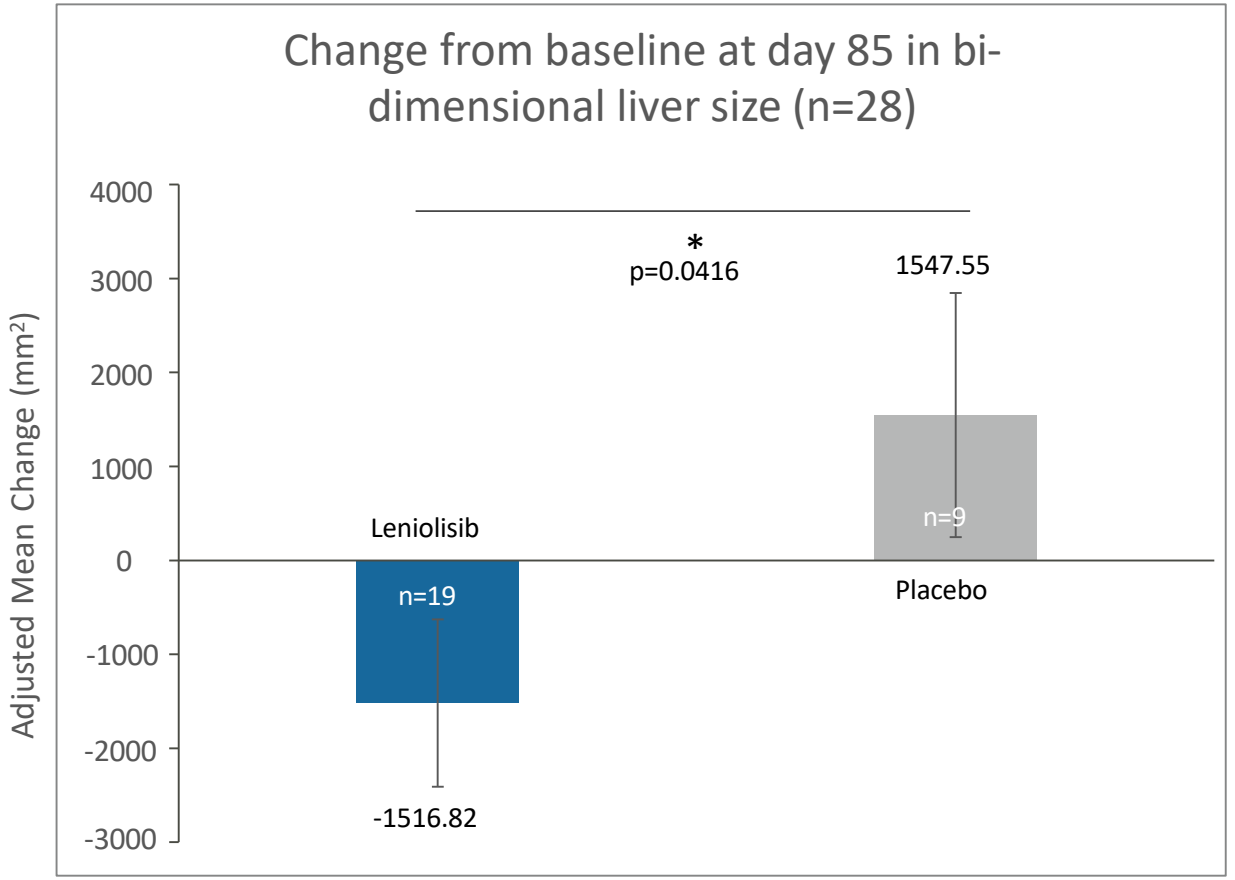
*Data were analyzed using an ANCOVA model with treatment as a fixed effect and baseline as a covariate. Use of glucocorticoids and IVIG at baseline were both included as categorical (Yes/No) covariates. *Baseline* is defined as the arithmetic mean of the baseline and Day 1 values when both are available, 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.

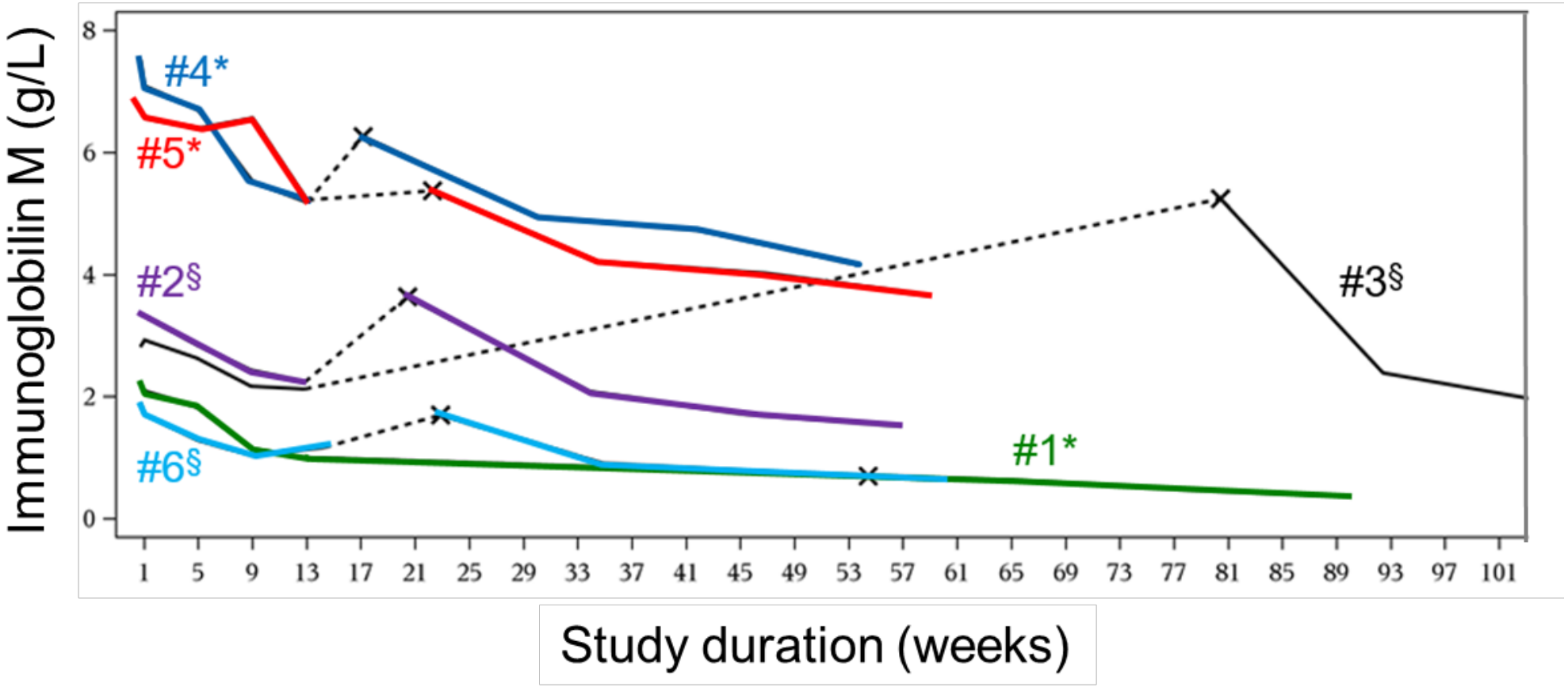
Leniolisib over three months was well tolerated

	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.

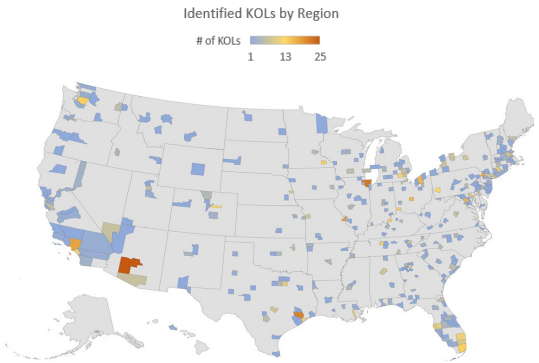
Launch preparations: Uncovering “APDS”

US targeted patient identification strategy

Pharming

1.

BUILD APDS NETWORK



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

2.

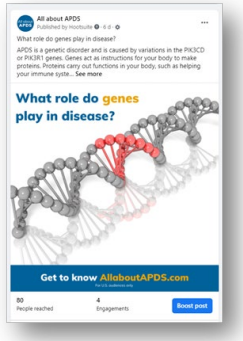
ANALYTICS & AI

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

3.

OUTREACH & EDUCATION

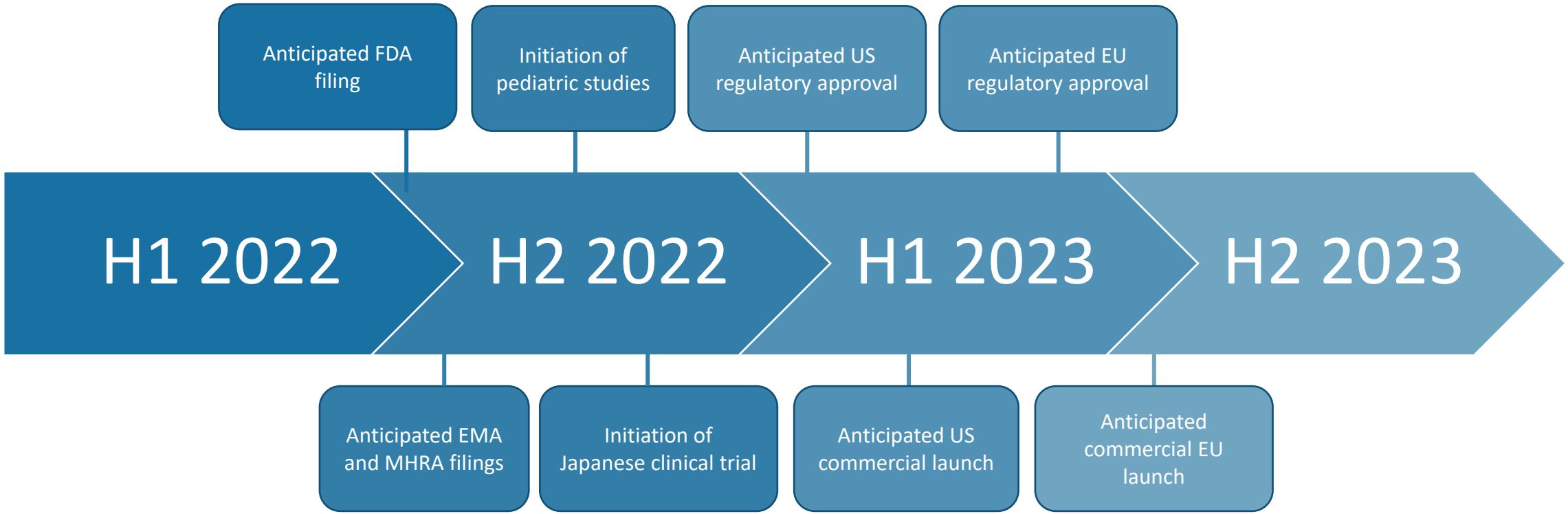
navigateAPDS Genetic Testing by Pharming



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.

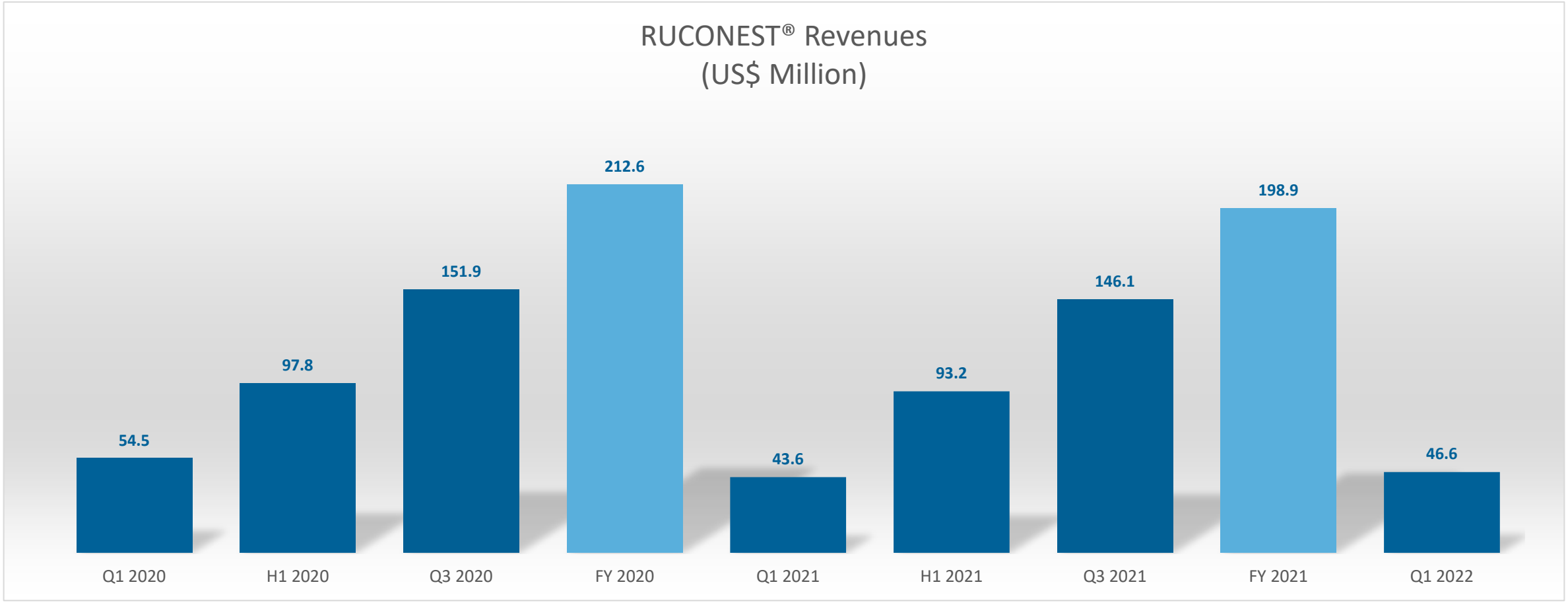
Leniolisib is an investigational new drug that has not been approved for any use.

Financial highlights from Q1 2022: Building a sustainable business (1/5)

Pharming

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 from Q1 2022: Building a sustainable business (2/5)

Pharming

REVENUE FROM SALES

- US revenues increased by 7% to US$45.3 million compared to Q1 2021
  - Increase in the number of patients treated partly offset tighter inventory management at larger specialty pharmacies.

GROSS PROFIT

- Gross profit increased by 8% to US$41.7 million (Q1 2021: US$38.7 million), mainly due to growth in revenues

Financial highlights from Q1 2022: Building a sustainable business (3/5)

Pharming

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

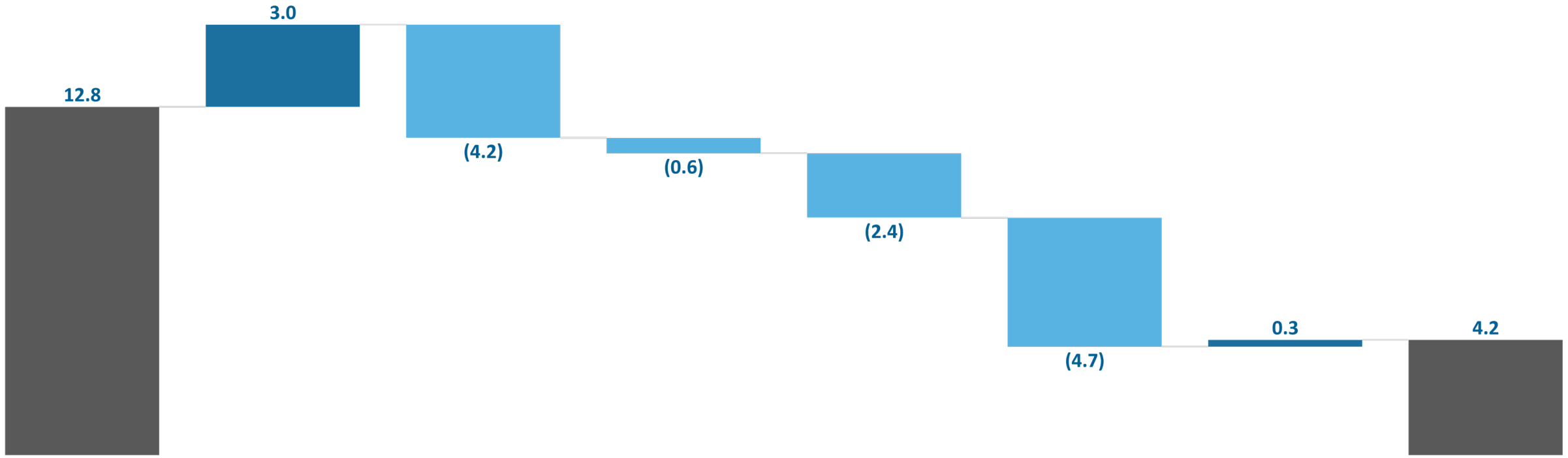
- Net profit of US$3.5 million decreased 59% (Q1 2021: US$8.5 million). The decrease was caused as a result of a significant decrease in finance income from US$6.6 million in Q1 2021 to US$1.8 million in Q1 2022, mainly due to more favorable exchange rate gains in Q1 2021
  - Remainder of the decrease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

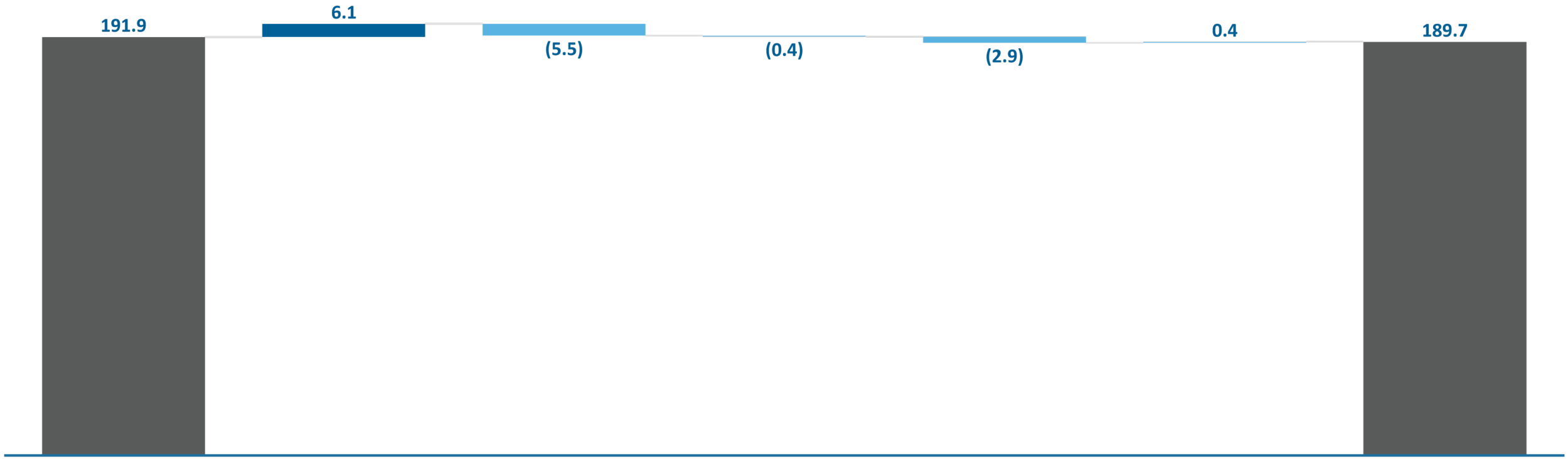
Financial highlights from Q1 2022: Profit before tax Q1 2021 – Q1 2022 (4/5)

(US$ Million)



Q1 2022: Cashflow 1 January 2022 – 31 March 2022 (5/5)

(US$ Million)



Outlook

- 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 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

Sijmen de Vries
Chief Executive Officer

Jeroen Wakkerman
Chief Financial Officer

Anurag Relan
Chief Medical Officer

www.pharming.com

investor@pharming.com

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

Pharming

Amounts in US$ '000	YTD 2022	YTD 2021
Revenues	**46,617**	**43,564**
Costs of sales	**(4,877)**	**(4,843)**
Gross profit	**41,740**	**38,721**
Other income	**873**	**259**
Research and development	(14,863)	(10,700)
General and administrative	(7,728)	(7,161)
Marketing and sales	(17,197)	(14,836)
Other Operating Costs	**(39,788)**	**(32,697)**
Operating profit	**2,825**	**6,283**
Fair value gain (loss) on revaluation derivatives	—	30
Other finance income	3,228	8,159
Other finance expenses	(1,379)	(1,598)
Finance result, net	**1,849**	**6,591**
Share of net profits in associates using the equity method	**(441)**	**(82)**
Profit before tax	**4,233**	**12,792**
Income tax expense	(772)	(4,269)
Profit for the year	**3,461**	**8,523**
Basic earnings per share (US$)	0.005	0.013
Diluted earnings per share (US$)	0.005	0.013

Balance sheet – assets

Amounts in US$ '000	March 31, 2022	December 31, 2021
Non-current assets		
Intangible assets	81,321	83,834
Property, plant and equipment	12,465	13,222
Right-of-use assets	19,432	19,943
Long-term prepayments	238	194
Deferred tax assets	20,194	21,216
Investment accounted for using the equity method	6,629	7,201
Investments in equity instruments designated as at FVTOCI	774	1,449
Restricted cash	797	812
Total non-current assets	**141,850**	**147,871**
Current assets		
Inventories	29,607	27,310
Trade and other receivables	31,445	29,983
Restricted cash	222	227
Cash and cash equivalents	189,674	191,924
Total current assets	**250,948**	**249,444**
Total assets	**392,798**	**397,315**

Balance sheet – liabilities

Equity		
Share capital	7,468	7,429
Share premium	457,961	455,254
Legal reserves	(910)	3,400
Accumulated deficit	(270,498)	(273,167)
Shareholders' equity	**194,021**	**192,916**
Non-current liabilities		
Convertible bonds	135,564	139,007
Lease liabilities	17,900	18,456
Other financial liabilities	162	165
Total non-current liabilities	**153,626**	**157,628**
Current liabilities		
Convertible bonds	1,844	1,879
Trade and other payables	40,827	42,473
Lease liabilities	2,480	2,419
Other financial liabilities	—	—
Total current liabilities	**45,151**	**46,771**
Total equity and liabilities	**392,798**	**397,315**

Cash flow (1/2)

Amounts in US$'000	YTD 2022	YTD 2021
Profit before tax	**4,233**	**12,792**
Non-cash adjustments:		
Depreciation, amortization, impairment of non-current assets	2,190	2,063
Equity settled share based payments	1,070	1,909
Fair value gain (loss) loss on revaluation of derivatives	—	(30)
Other finance income	(3,228)	(8,159)
Other finance expenses	1,379	1,598
Share of net profits in associates using the equity method	441	(82)
Other	—	(1,094)
Operating cash flows before changes in working capital	**6,085**	**8,997**
Changes in working capital:		
Inventories	(2,297)	(608)
Trade and other receivables	(1,462)	2,961
Payables and other current liabilities	(1,645)	(4,006)
Restricted cash	(20)	(321)
Total changes in working capital	**(5,424)**	**(1,974)**
Interest received (paid)	(52)	38
Income taxes paid	—	—
Net cash flows generated from (used in) operating activities	**609**	**7,061**

Cash flow (2/2)

Capital expenditure for property, plant and equipment	(208)	(1,956)
Investment intangible assets	(167)	(460)
Investment associate	—	398
Investment in equity instruments designated as at FVTOCI	—	—
Acquisition of license	—	(547)
Net cash flows used in investing activities	**(374)**	**(2,565)**
Payment on contingent consideration	—	—
Payment of lease liabilities	(807)	(554)
Interests on loans	(2,100)	(2,500)
Proceeds of equity and warrants	18	674
Net cash flows generated from (used in) financing activities	**(2,889)**	**(2,380)**
Increase (decrease) of cash	**(2,654)**	**2,116**
Exchange rate effects	404	(650)
Cash and cash equivalents at 1 January	191,924	205,159
Total Cash and cash equivalents at 31 March	**189,674**	**206,625**