Q1 2023 Results
Investor presentation

NOVARTIS | Reimagining Medicine

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Vas Narasimhan, M.D.

Chief Executive Officer

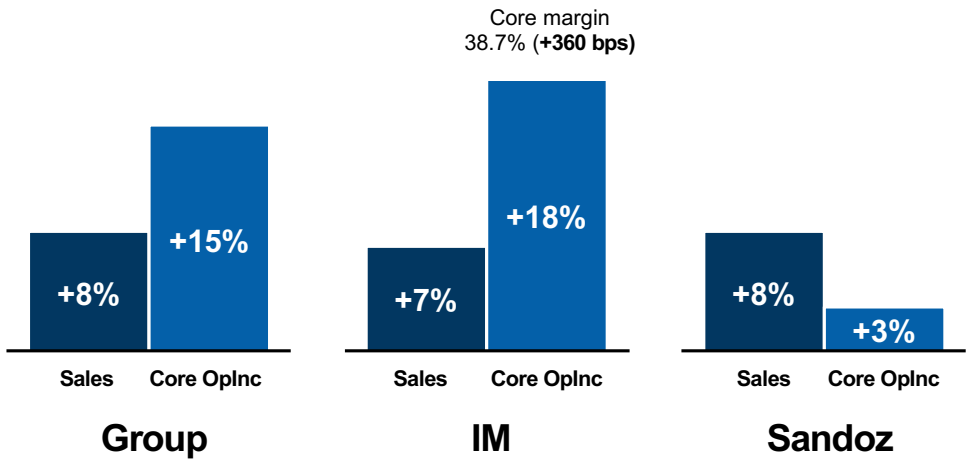
Company overview

Q1 SUMMARY

Novartis delivers strong sales growth, robust margin expansion and major innovation milestones; raising FY 2023 guidance

Growth and Productivity

Q1, % cc



Innovation

Kisqali® NATALEE Ph3 met primary endpoint in broad early breast cancer population

Cosentyx® demonstrated durable efficacy up to 52 weeks in Hidradenitis suppurativa (HS)

Entresto® positive CHMP opinion in pediatric HF

RLT platform i) Acquisition of FAP-targeting asset[1] and ii) research collaboration on bicyclic peptides[2]

Operations

Millburn and Zaragoza approved for Pluvicto®

Constant currencies (cc), core results are non-IFRS measures; explanation can be found on page 35 of Condensed Interim Financial Report. Unless otherwise noted, all growth rates refer to same period in PY. OpInc – operating income. IM – Innovative Medicines division. LMIC – low and middle income countries. HIC – high income countries. CHMP – Committee for Human Medicinal Products. HF –heart failure. FAP – fibroblast activation protein.
1. Clovis Oncology. 2. Bicycle Therapeutics.

INNOVATION

Key 2023 readouts for high-value medicines on track

Key assets* with submission enabling readouts in 2023

Kisqali® ●●●

Ph3 NATALEE trial in adjuvant breast cancer testing broad patient population (anatomical stage II and III[1]), with final analysis expected in **H2 2023**

Primary endpoint met at interim analysis

Pluvicto® ●●

PSMAfore trial in mCRPC (post-ARDT, pre-taxane) positive readout; detailed data presentation planned in **H2 2023**

FDA regulatory submission planned in **H2 2023**

Iptacopan ●●●

APPOINT-PNH trial in treatment-naive patients positive readout; detailed data presentation in **2023**

Data at EBMT

PNH FDA filing planned in **H1 2023**

APPLAUSE-IgAN Ph3 readout[2] planned in **H2 2023**

APPEAR-C3G Ph3 readout planned in **H2 2023**

*Unprobabilized peak sales of all asset indications in late-stage development: ● > USD 1bn ●● > USD 2bn ●●● > USD 3bn

mCRPC – metastatic castration resistant prostate cancer. ARDT – androgen receptor directed therapy. 1. Based on AJCC prognostic staging. 2. 9 months analysis potentially supporting US Subpart H filing.

INNOVATION

Submission enabling readouts expected to increase in 2024-2025 timeframe

Selected key assets* with submission enabling readouts in 2024-2025

Remibrutinib ●●

CSU
Primary analysis[1] in **H2 2023**
Final (52 weeks) readout and submission in **2024**

Scemblix® ●●

1L CML-CP
Readout and submission in **2024**

Accelerated submission timeline

Pluvicto® ●●

mHSPC
Readout and submission in **2024**

OAV-101 ●●

SMA IT
Readout in **2024**; submission in **2025**

Pelacarsen ●●●

CVRR
Readout and submission in **2025**

Ianalumab ●●●

1L and 2L ITP readouts in **2025** with submission in **2026**
Additional hematology and immunology indications **2026+**

Iptacopan ●●●

Additional readouts/submissions in **2025/2026+**

*Unprobabilized peak sales of all asset indications in late-stage development: ● > USD 1bn ●● > USD 2bn ●●● > USD 3bn

1. Double blind treatment period of 24 weeks with primary analysis at 12 weeks.

INNOVATION

Kisqali® NATALEE study met its primary endpoint demonstrating clinically meaningful iDFS benefit in broad eBC population

Kisqali® **(400mg)** plus ET significantly **reduced the risk of disease recurrence** (vs. standard ET alone)

Data to be presented at an **upcoming medical meeting**

30-60% of people with stage II and III eBC treated with ET alone **remain at risk of BC recurrence**

Consistent benefit in a broad population of stage II and III eBC patients at risk of recurrence, including those with no nodal involvement

Worldwide regulatory **submissions on track for H2 2023**

400mg dose used to **reduce dose-dependent AEs** given **tolerability** profile of treatment is **critical in early breast cancer**

eBC – early breast cancer. ET – endocrine therapy. iDFS – invasive disease free survival.

INNOVATION

Kisqali® NATALEE unique in covering a broad population of eBC

Kisqali eBC opportunity

Multibillion USD[1] ●●●

Incident population (estimated)[3]

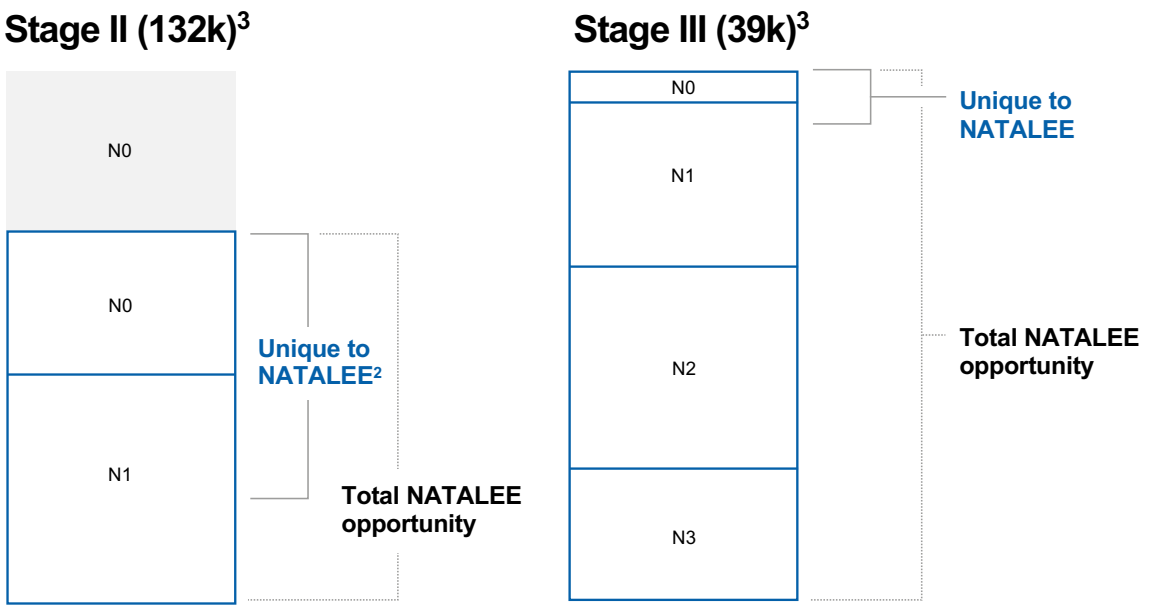
	US	EU5
Stage II	66k	66k
Stage III	15k	24k
Stage IV (metastatic)	32k	32k

NATALEE population

Covers ~70% of Stage II, 100% of Stage III

~2-3x patient opportunity vs. monarchE

NATALEE covers broad population in eBC

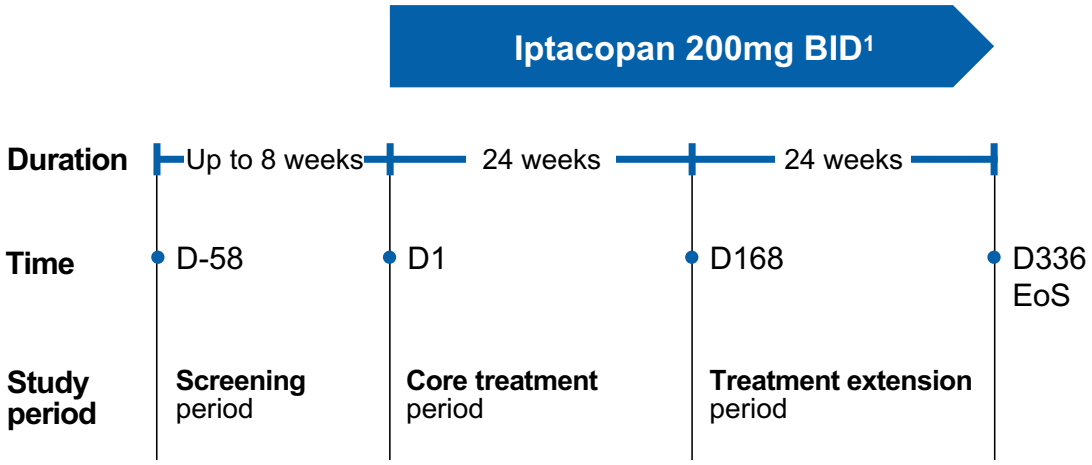


eBC – early breast cancer. ET – endocrine therapy. iDFS – invasive disease-free survival. 1. Unprobabilized peak sales. 2. Under stage II: N0, T0N1 is excluded; T2N0 only if G3, or G2 with Ki67≥20% or high risk on Oncotype DX / Prosigna / MammaPrint / EndoPredict. 3. Estimated incidence US + EU5. Sources: DRG (US) and Kantar (EU5). TNM and grade information based on SEER AJCC 8th Incidence Report.

INNOVATION

Iptacopan APPOINT-PNH data showed clinically meaningful increases in Hb levels for treatment-naive adult patients with PNH

APPOINT-PNH: Single-arm Ph3 trial in adult patients with PNH with hemolysis (LDH > 1.5x ULN) and anemia (Hb < 10g/dL) naive to complement inhibitors



Study status

Met primary endpoint of proportion of patients achieving a sustained increase in Hb of ≥ 2g/dL, in the absence of transfusions, at 24 weeks

Safety profile consistent with previously reported data

Data to be presented at EBMT April 26, 2023

Hb – hemoglobin. LDH – lactate dehydrogenase. ULN – upper limit of normal. PNH – paroxysmal nocturnal hemoglobinuria. EBMT – European society for blood and marrow transplantation. 1. BID – twice daily.

INNOVATION

Strengthening radioligand therapy pipeline; advancing multiple assets in clinical development

Recent business development in RLT

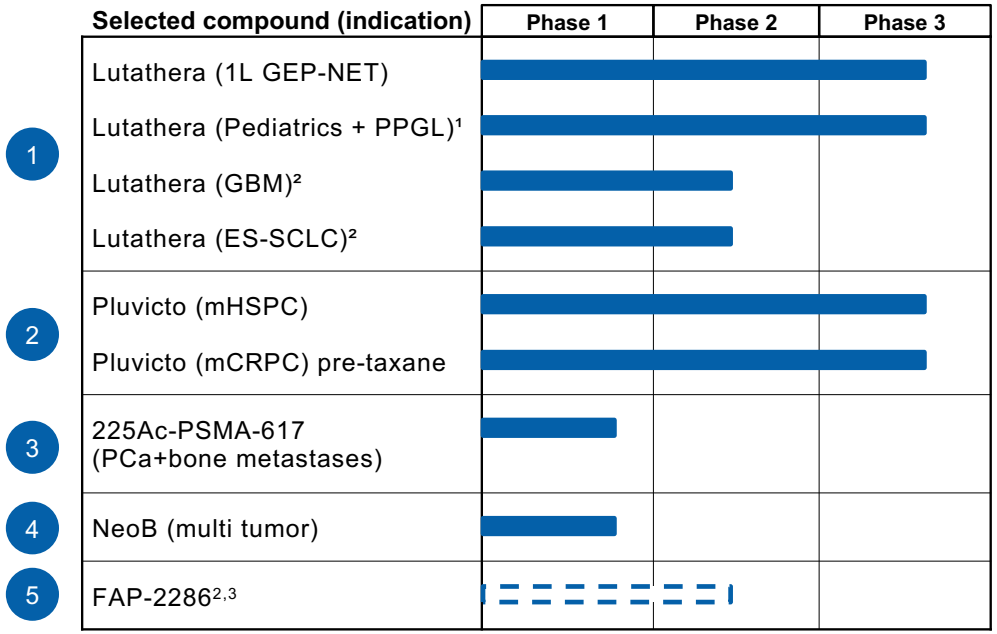
Discovery collaboration with Bicycle Therapeutics

- Bicycle® platform employs constrained cyclic peptides
  - Provides variety of structural shapes/chemical diversity
  - May broaden the tractable target space (key differentiator from other peptide platforms)
- Supplements our existing vector discovery platforms

Acquisition of FAP-2286 (Clovis Oncology)

- Fibroblast Activation Protein (FAP) represents a promising RLT target in PDAC, CRC, BC, NSCLC
  - Frequently expressed on cancer-associated fibroblasts across cancers
- FAP-2286, currently developed in Ph1/2
  - Showed first signs of efficacy/favorable safety profile
- Potential to be first-in-class, transformative RLT

Current RLT pipeline



PDAC – pancreatic ductal adenocarcinoma. CRC – colorectal cancer. BC – breast cancer. NSCLC – non-small cell lung cancer. GEP-NET – gastroenteropancreatic neuroendocrine tumor. ES-SCLC – extensive stage small cell lung cancer. mHSPC – metastatic hormone sensitive prostate cancer. mCRPC – metastatic castration resistant prostate cancer. 1. PPGL, pheochromocytomas and paragangliomas, are an exploratory cohort of NETTER-P. 2. Phase 1/2. 3. Being integrated in the NVS pipeline.

GROWTH

Q1 growth driven by strong performance from Entresto®, Pluvicto®, Kesimpta® and Kisqali®

Q1 sales

	Sales USD million	Growth vs. PY USD million	Growth vs. PY cc
Entresto	1,399	306	32%
PLUVICTO	211	209	nm
Kesimpta (ofatumumab)	384	189	100%
KISQALI ribociclib	415	176	81%
SCEMBLIX (asciminib)	76	51	202%
LEQVIO	64	50	nm
PROMACTA (eltrombopag)	547	56	15%
Tafinlar + Mekinist	458	55	18%
ILARIS (canakinumab)	328	43	19%
PIQRAY (alpelisib) tablets	116	43	61%
JAKAVI ruxolitinib	414	25	13%

Strong growth (+67% cc); expected to continue

Constant currencies (cc) is a non-IFRS measure; explanation of non-IFRS measures can be found on page 35 of Condensed Interim Financial Report. Unless otherwise noted, all growth rates refer to same period in PY.
nm – not meaningful.

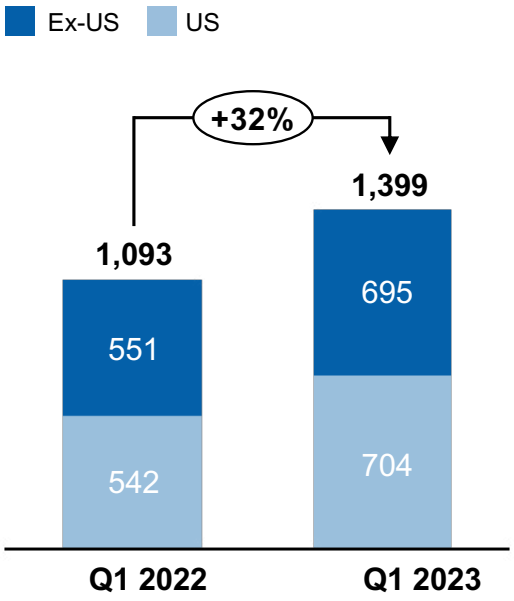
GROWTH

Entresto® delivering strong double-digit growth in all geographies

Entresto®

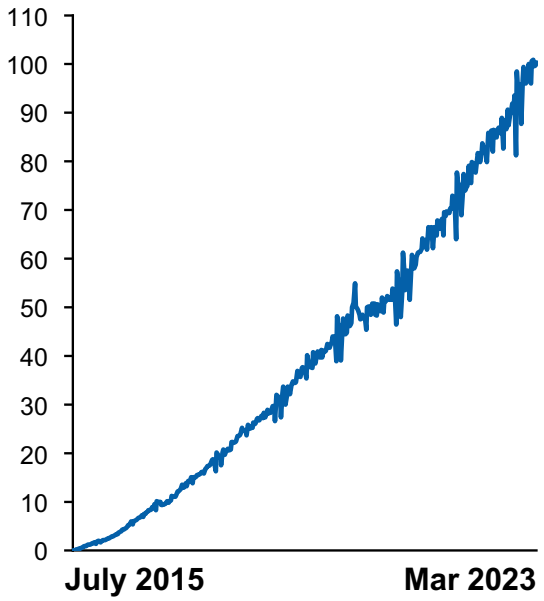
Sales evolution

USD m, % cc



US weekly TRx[1]

Total prescriptions (000)



Strong Q1 momentum, outpacing market[2]

US: NBRx **+30%** vs PY, ~1.3m TRx in Q1[1]

EU: Continued growth in HFrEF

China/Japan: Significant contribution from HTN[3]

Confidence in future growth[4]

Expect **further penetration** in HFrEF (2/3 eligible patients still on prior SoC)

Robust guideline position[5] **(US/EU)**

CHMP positive opinion for pediatric HF[6]

China/Japan: Launch momentum in HTN, inclusion in 2023 China HTN guideline as 1L option

TRx – total prescriptions. NBRx – new to brand prescriptions. HFrEF – heart failure with reduced ejection fraction. HF – heart failure. HTN – hypertension. SOC – standard of care. 1. IQVIA National Prescription Audit. 2. CHF market basket includes ACEi, ARB, SGLT2i, Entresto. Data refers to US. 3. Approved indications differ by geography. Examples include "indicated to reduce the risk of cardiovascular death and hospitalization for HF in adult patients with CHF. Benefits are most clearly evident in patients with LVEF below normal." (US), HFrEF (EU), HFrEF and HTN (China) and CHF and HTN (JP). HTN is not an approved indication in the US. 4. For forecasting purposes, we assume no generic entry in US before 2025. 5. AHA/ACC/HFSA/ESC. 6. If approved, pediatric indication would support extension of the regulatory data protection to November 2026 in EU.

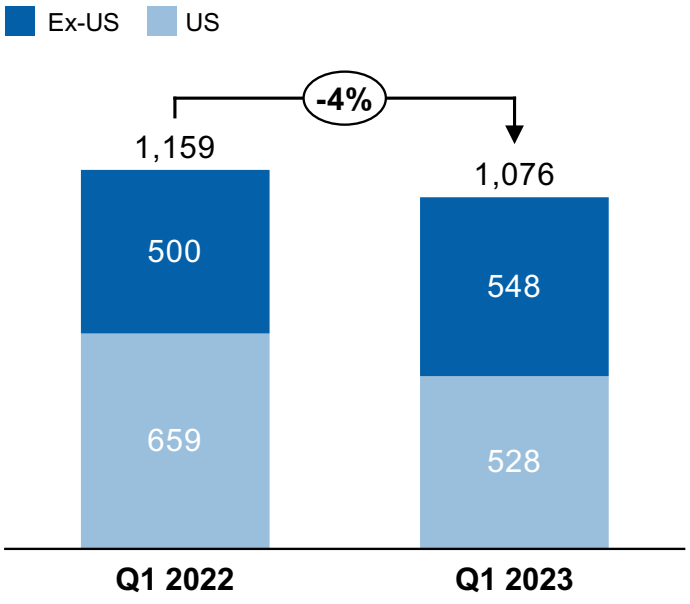
GROWTH

Cosentyx® ex-US growth offset by US decline. Global FY sales expected broadly in line with PY

Cosentyx®

Sales evolution

USD m, % cc



Q1 performance

US: Demand growth offset by revenue deductions (incl. PY base impact)

Ex-US: Strong growth in core indications

China: Outperforming market with double-digit growth post-COVID

2023: Expect FY sales broadly in line with PY

(H1 decline and H2 growth)

Future growth mainly driven by life cycle management

EU: CHMP opinion for HS expected Q2

US: HS/ IV approvals expected H2

Lupus Nephritis and Giant Cell Arthritis Phase 3 trials **on track**

New Phase 3 trials initiated in Polymyalgia Rheumatica and Rotator Cuff Tendinopathy

CHMP – Committee for Human Medicinal Products. HS – hidradenitis suppurativa. IV – intravenous.

GROWTH

Cosentyx® HS demonstrated durable efficacy, sustained up to 1 year

Cosentyx®

HS unmet need

Lesions and **abscesses** in sensitive areas of the body

~97% patients suffer from pain[1]

~95% eligible patients not on biologic[2]

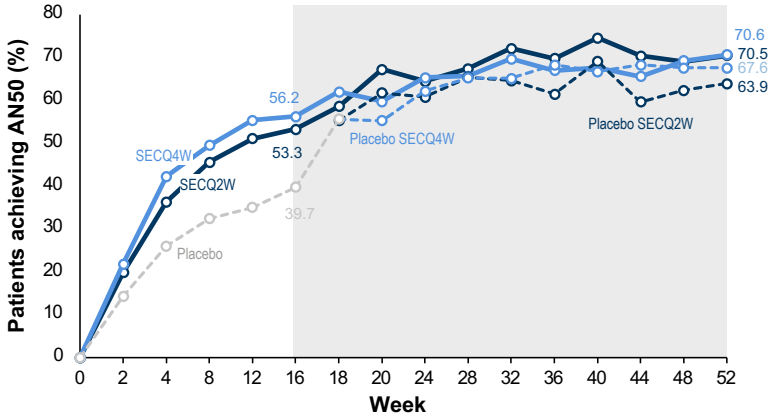
~50% biologic treated patients lose response[3]

Cosentyx opportunity

~400k addressable patients in US and EU[4]

Cosentyx Ph3 data**

Durable efficacy sustained to 1 year



>70% with at least a 50% reduction in total abscess & inflammatory nodule count[5]

≥70% flare free[5]

>65% with pain relief[6]

Fast and lasting **QoL** improvement[5]

Safety consistent with well-established* profile[7,5]

Well tolerated | Infrequent SAEs | Candidiasis uncommon[8] | Low immunogenicity

See page 94 for references 1-8. HS – hidradenitis suppurativa. QoL – quality of life. SAE – serious adverse event. *Refers to approved indications. **HS indication currently under regulatory review.

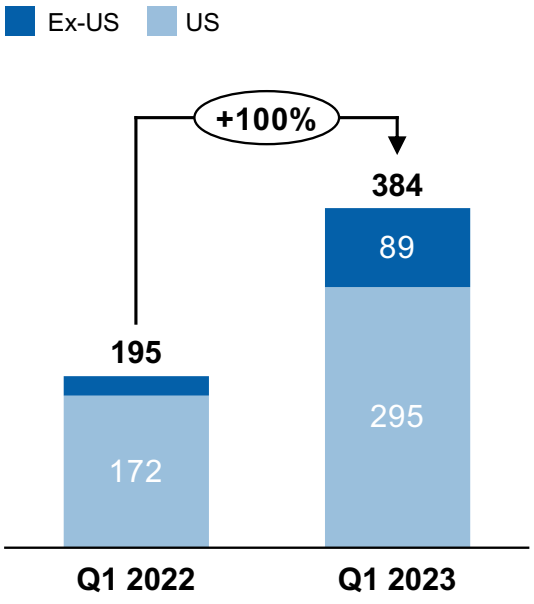
GROWTH

Kesimpta® continues strong launch trajectory doubling sales vs. PY

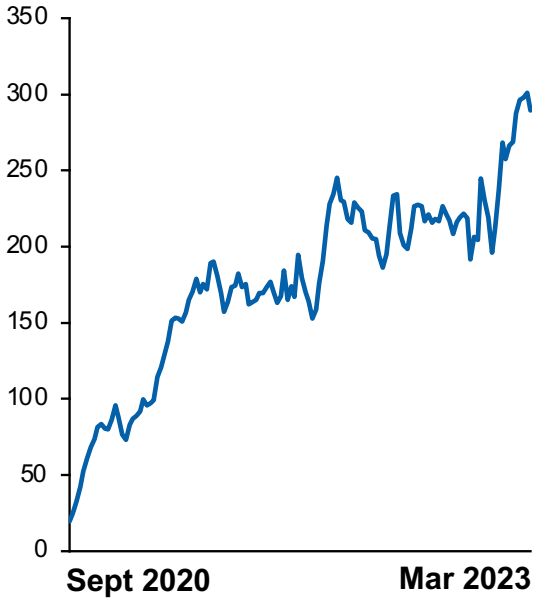
Kesimpta®

Sales evolution

USD m, % cc



US weekly NBRx[1]



Global sales +100% (cc)[2]

US: Growing faster than market[1,2]

TRx **+89%** vs. PY (market +1%)

NBRx **+60%** vs. PY (market -8%)

B-cell NBRx share **~50%** of MS market

Kesimpta® NBRx share **~14%** of MS market

Europe: Strong launch momentum[3]

65% of population with access to Kesimpta

>18k patients treated, thereof >1/3 naive patients

Confident in future growth

Significant room to grow: only **40%** of MS patients on B-Cell therapy in US[1,2]

Compelling product profile: 1 minute a month dosing from home/anywhere[4]; 5 year efficacy[5] and safety data[6,7]

See page 94 for references 1-7. TRx – total prescriptions. NBRx – new to brand prescription.

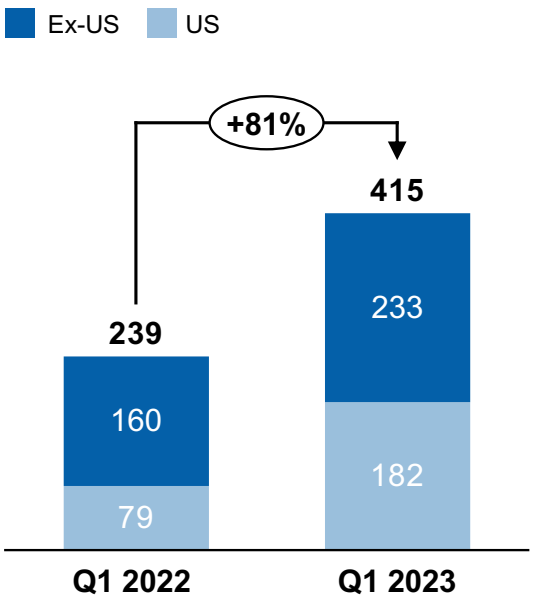
GROWTH

Kisqali® gaining momentum globally, with increasing recognition of its differentiated profile

KISQALI®

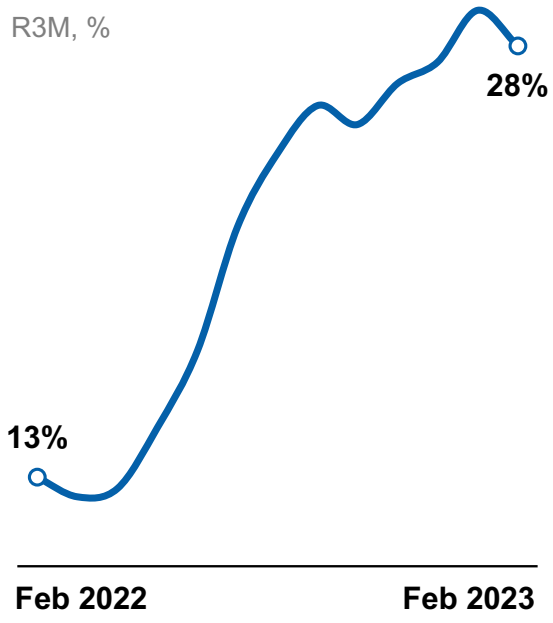
Sales evolution

USD m, % cc



US metastatic BC NBRx share[1]

R3M, %



Q1 sales USD 415 million, **+81% cc**

US NBRx[1] share ~doubled vs. PY to 28%

EU5 1L mBC NBRx share up to 38% (Q4'22) vs. 32% (in Q3'22)

Favorable NCCN[2] guidelines positioning Kisqali® as only Category 1 treatment for 1L mBC with AI

Positive readout of Ph3 NATALEE study in eBC; data to be presented at upcoming congress

mBC – metastatic breast cancer. NBRx – new to brand prescription. R3M – rolling 3 months. eBC – early breast cancer. NCCN – national comprehensive cancer network. AI – aromatase inhibitor. 1. Of CDK4/6 mBC market, US Q1 R3M. 2. NCCN Guidelines updated as of 27-Jan-2023.

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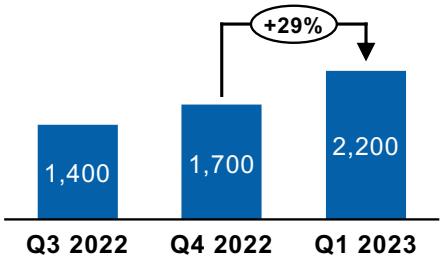
GROWTH

Leqvio® adoption expanding as we progress the launch

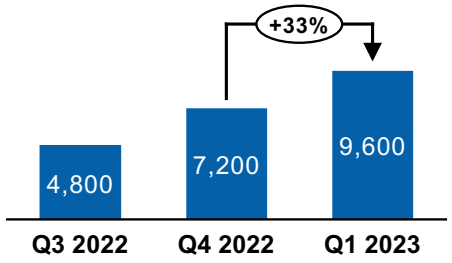
LEQVIO®

Addressing non-clinical barriers in US

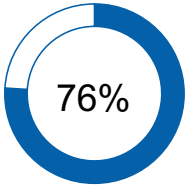
Adoption
Facilities[1] having **ordered Leqvio®**



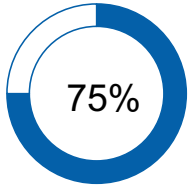
Adoption
HCPs[2] with **Leqvio® experience**



Access
Patients covered **at or near label**[3]

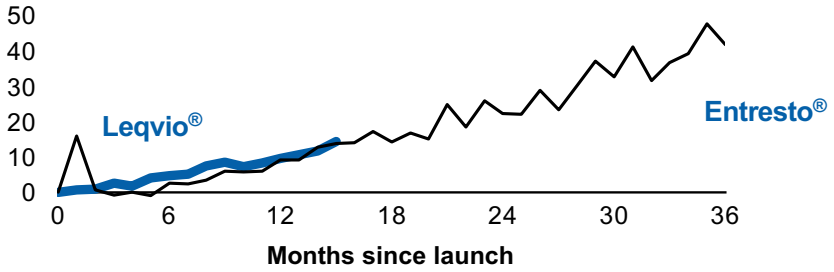


Adherence
Patients coming for **2nd dose* within <95 days**[4]

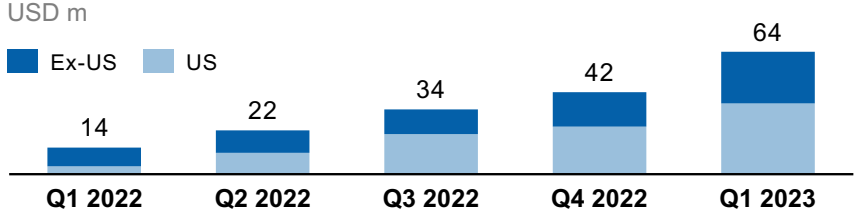


US Leqvio® launch tracking Entresto®

US sales evolution
USD m



Global sales evolution
USD m



HCP – healthcare professional. 1. Either an alternate site of care or a physician practice. 2. Either prescribe Leqvio® to a patient based on service center data, data on file or have ordered through Free Trial Offer program. 3. As of April 10, 2023. 4. Refers to average duration in between doses. Based on IQVIA and data shared by infusion management and ambulatory infusion center companies. *Leqvio® is administered initially, again at 3 months, and then once every 6 months. Novartis has obtained global rights to develop, manufacture and commercialize Leqvio® under a license agreement with Alnylam Pharmaceuticals.

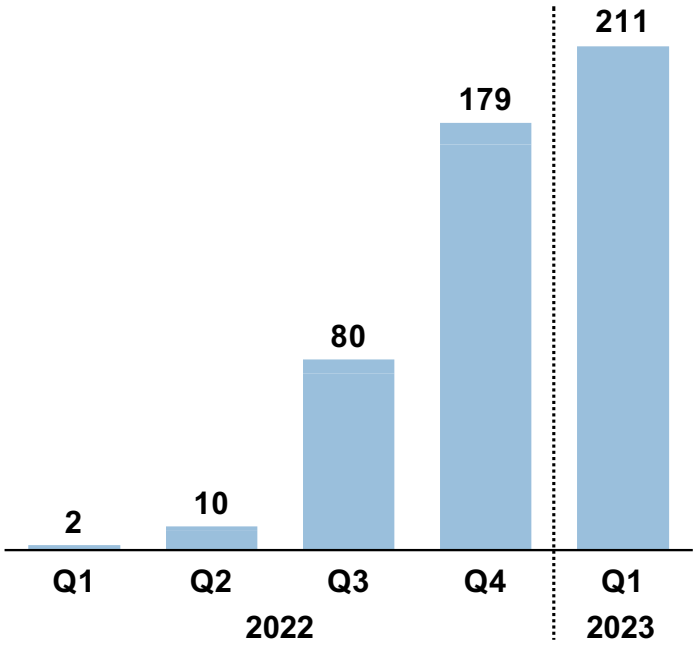
GROWTH

Pluvicto® uptake reflects strong benefit/risk profile and unmet need in post-taxane mCRPC

PLUVICTO™

Sales evolution

Global sales, USD m



Q1 sales USD 211 million (mostly US)

Q2 sales expected broadly in line with Q1

200 unique accounts in US currently treating with Pluvicto®

PSMAfore pre-taxane study (which met its primary endpoint of rPFS) expected to be presented in H2 2023

FDA **submission for PSMAfore** including OS data is **planned for H2 2023** as aligned with FDA

Millburn facility approved to support US launch; Zaragoza approved for EU

mCRPC – metastatic castration-resistant prostate cancer. rPFS – radiographic progression free survival. OS – overall survival.

GROWTH

Millburn approved for Pluvicto® commercial supply in US; Zaragoza approved for EU

PLUVICTO™

Expected commercial coverage by end 2023

Manufacturing site:	Ivrea	Millburn	Indianapolis	Zaragoza
US	✓ Approved	✓ Approved	FDA filing in preparation	-
EU	✓ Approved	-	-	✓ Approved
ROW	✓ Approved[1]	✓ Approved[2]	In preparation[2]	In preparation

Millburn to ramp up gradually; expected to contribute meaningfully to supply and sales in **Q3** after anticipated approval of additional lines

Ivrea will continue to supply the US market in addition to EU

Zaragoza also approved to supply EU

Targeting capacity of at least 250k doses annually for 2024+[3]

1. Currently approved for Canada, Switzerland and UK; further ROW submissions ongoing. 2. Canada. 3. Total production across all RLTs.

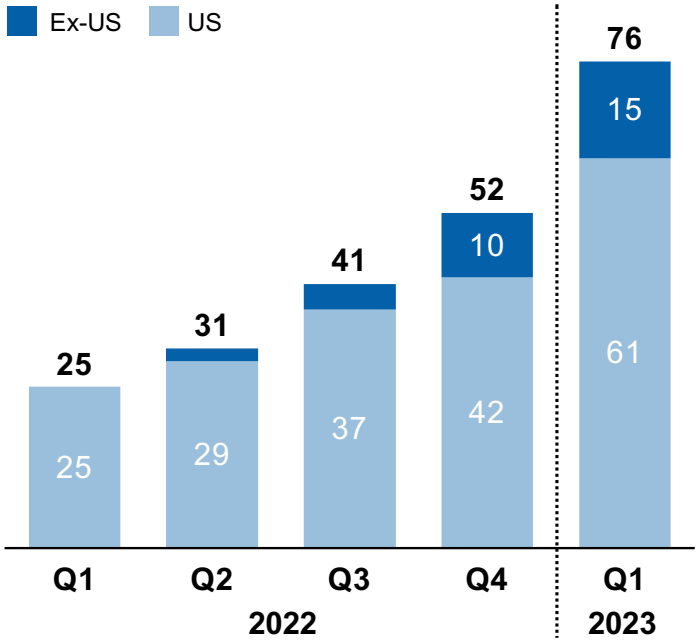
GROWTH

Scemblix® maintains strong launch momentum

SCEMBLIX® (asciminib) 20 mg, 40 mg tablets

Sales evolution

USD m



Q1 sales **USD 76 million; US NBRx share at 32%**[1]

Sales driven by patients resistant/intolerant to other TKIs

Global rollout ongoing with approval in 46 countries; access pathways in 19, negotiations ongoing in 30+

Increasing recognition of **efficacy and tolerability benefit:** G-BA granted the highest ever rating for a medicine in CML

ASC4FIRST (**1L registrational** study) completed enrollment ahead of plan, readout and **filing expected 2024**

TKI – tyrosine kinase inhibitor. NBRx – new to brand prescription. G-BA – German national payer (Gemeinsamer Bundesausschuss). 1. IQVIA: US Jan 2023 rolling three months 3L+ new patient start share.

Harry Kirsch
Chief Financial Officer

Financial review and 2023 guidance

FINANCIAL PROFILE

Strong top and bottom line growth in Q1

Group[1] USD million	Q1 2023	Change vs. PY % USD	Change vs. PY % cc
Net Sales	12,953	3	8
Core Operating income	4,413	8	15
Operating income	2,856	0	9
Net income	2,294	3	14
Core EPS (USD)	1.71	17	25
EPS (USD)	1.09	9	20
Free Cash Flow	2,720	95	

1. Core results, constant currencies and free cash flow are non-IFRS measures. Further details regarding non-IFRS measures can be found starting on page 35 of the Condensed Interim Financial Report.

FINANCIAL PROFILE

Continuing core margin improvements for Group driven by IM

	Q1 2023			
	Net sales change vs. PY[1] % cc	**Core operating income** change vs. PY[1] % cc	**Core margin[1]** %	**Core margin** change vs. PY[1] %pts cc
Innovative Medicines	7	18	38.7	3.6
Sandoz	8	3	21.1	-1.0
Group	**8**	**15**	**34.1**	**2.2**

1. Constant currencies (cc), core results are non-IFRS measures. An explanation of non-IFRS measures can be found on page 35 of the Condensed Interim Financial Report.

FINANCIAL PROFILE

Raising 2023 guidance for Novartis excluding and including Sandoz

Expected, barring unforeseen events; growth vs. PY in cc

		Previous guidance
Innovative Medicines (IM)	Sales expected to **grow mid single digit**	(from low-to-mid)
	Core OpInc expected to **grow high single digit to low double digit**	(from mid-to-high)
Novartis ex. Sandoz (IM + Corporate)	Sales expected to **grow mid single digit**	(from low-to-mid)
	Core OpInc expected to **grow high single digit to low double digit**	(from mid-to-high)
Novartis incl. Sandoz (IM + Sandoz + Corporate)[1]	Sales expected to **grow mid single digit**	(from low-to-mid)
	Core OpInc expected to **grow high single digit**	(from mid)

Key assumptions:

- Our guidance assumes that no Sandostatin® LAR generics enter in the US in 2023
- We continue to expect that the planned Sandoz spin-off is completed in H2 2023

1. Novartis Group guidance, assuming Sandoz would remain within the Group for the entire FY 2023.

FINANCIAL PROFILE

Raising Sandoz 2023 top line guidance

Expected, barring unforeseen events; growth vs. PY in cc

		Previous guidance
2023	Sales expected to **grow mid single digit**	(Low-to-mid)
	Core OpInc expected to **decline low double digit** reflecting required stand-up investments to transition Sandoz to a separate company and continued inflationary pressures	
Mid-term	Sales expected to **grow low-to-mid single digit CAGR** Core OpInc margin expected to **expand to mid 20s,** continuously progressing from the low 2023 base driven by continued sales growth and operational efficiencies	

Key assumptions:

- We continue to expect that the planned Sandoz spin-off is completed in H2 2023

Note: after completion of planned Sandoz spin-off, Core OpInc guidance will be expressed in terms of core EBITDA.

FINANCIAL PROFILE

Sandoz well positioned in a growing market; planned spin-off on track for H2

Q1 performance[1]

Sales USD 2.4bn **(+8%)**

Biopharma grew 17%; Retail 6%

Strong ex-US sales growth:

- EU: USD 1.4bn **(+16%)**
- RoW: USD 0.6bn (+4%)
- US: USD 0.4bn (-7%)

Core OpInc (+3%)

Continuing to deliver on biosimilar promise

Adalimumab HCF approved in US, Europe; launch starting in H2

Denosumab filing accepted (US)

Aflibercept Ph3 readout in H2

Announced **USD 400 million investment** for new biologics plant (Slovenia)

On track for planned Sandoz spin-off[2] in H2 2023

Capital Markets Day – June 8 (NYC) and June 12 (London)

Gilbert Ghostine appointed as Sandoz Chairman-Designate

Expected tax neutral for Novartis and majority of shareholders

HCF – high concentration formulation. 1. All growth rates in constant currencies (cc). 2. Transaction requires Novartis BoD and shareholder approval.

FINANCIAL PROFILE

Group Core OpInc to grow high single digit driven by business momentum and Pluvicto® manufacturing capacity expansion

2023 key drivers of core operating income (Group)

Vs. PY (cc) Illustrative

+ In-market growth drivers to continue growing strongly
+ Recent launches to further accelerate, Pluvicto® benefiting from manufacturing capacity expansion
+ China growth expected to accelerate benefiting from return to normal in H2
+ Simplified organizational model to deliver continued SG&A savings
+ Ongoing productivity programs

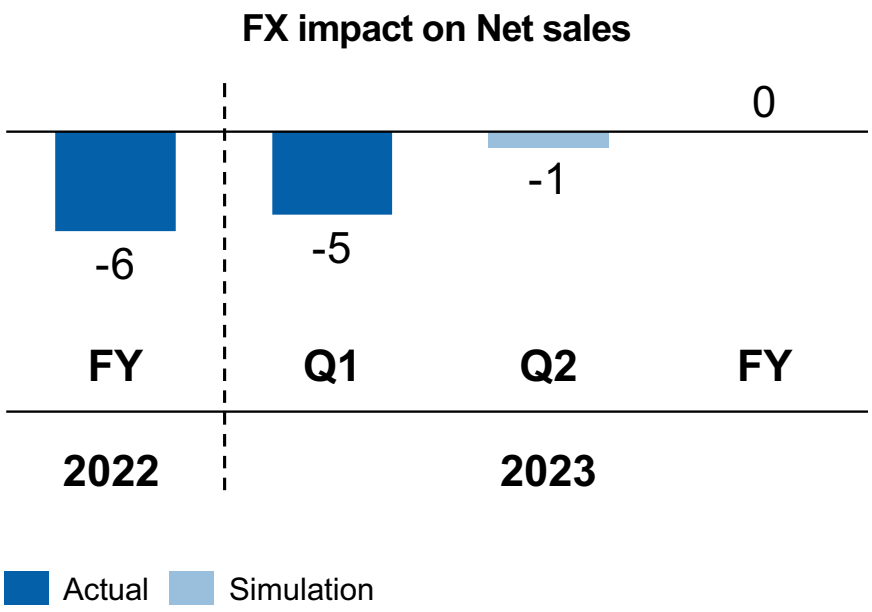
− Impact of inflation expected to continue
− Gx erosion expected to increase due to Gilenya® US and Lucentis® EU
− Stand-up investments to transition Sandoz to a standalone company

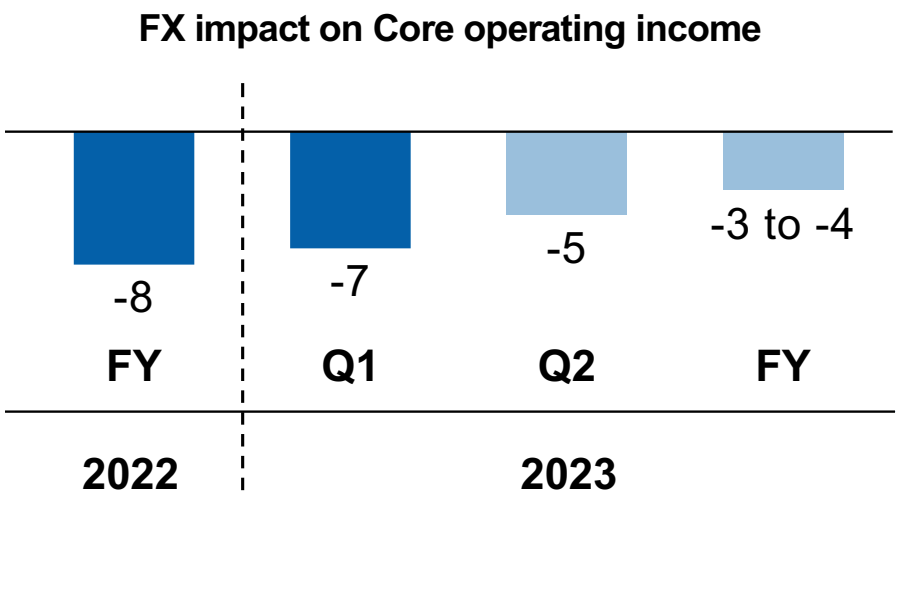
FINANCIAL PROFILE

Expected currency impact for full year 2023

Currency impact vs. PY

%pts, assuming late-April exchange rates prevail in 2023





Actual Simulation

Vas Narasimhan, M.D.

Chief Executive Officer

Conclusions

Strong start to 2023: Growth particularly driven by Entresto®, Kisqali® and Kesimpta®

Launches: Pluvicto® and Scemblix® continue strong trajectory, Leqvio® progresses steadily

Confidence in near- to mid-term growth: NATALEE Phase 3 positive readout, upcoming iptacopan data, and Pluvicto® in earlier lines of therapy

Raising guidance: Strong start and confidence in growth drivers allow to raise FY 2023 guidance

Appendix

INNOVATION

2023 expected key events

		H1 2023	H2 2023	Status update – as of end Q1
Regulatory decisions	**Cosentyx® HS**	EU	US	
	Cosentyx® 2ml AI	US		
	Cosentyx® IV		US	
	Leqvio® Hypercholesterolemia		JP, China	
Submissions	**Iptacopan PNH (US/EU/JP)**	US/EU	JP	
	Kisqali® HR+/HER2- BC (adj)		US	Filing expected in H2
	Pluvicto® mCRPC, pre-taxane (US)		US	
Readouts	**Kisqali® HR+/HER2- BC (adj)**		NATALEE Ph3 FIR	Primary endpoint met at interim analysis
	Iptacopan IgAN Ph3		APPLAUSE-IgAN Ph3	
	Iptacopan C3G Ph3		APPEAR-C3G Ph3	
Ph3 starts	**Iptacopan in IC-MPGN**		Ph3	
	Leqvio® CVRR primary prevention	Ph3		VICTORION-1P initiated
	Ianalumab in immune thrombocytopenia	Ph3		1L (VAYHIT1) and 2L (VAYHIT2) initiated
	Ianalumab in systemic lupus erythematosus	Ph3		SIRIUS-SLE 1 and 2 initiated

HS – hidradenitis suppurativa. PNH – paroxysmal nocturnal hemoglobinuria. mCRPC – metastatic castration-resistant prostate cancer. FIR – first interpretable results. IgAN – immunoglobulin A nephropathy. C3G – complement 3 Glomerulopathy. IC-MPGN – immune complex membranoproliferative glomerulonephritis.

Our pipeline projects at a glance

	Phase 1/2	Phase 3	Registration	Total
Innovative medicines	**81**	**46**	**7**	**134**
Solid Tumors	15	15	2	32
Hematology	18	8	0	26
Immunology	20	10	4	34
Neuroscience	5	5	0	10
Cardiovascular	6	6	1	13
Others	17	2	0	19
Ophthalmology	*4*	*1*	*0*	*5*
Respiratory & Allergy	*3*	*0*	*0*	*3*
Global Health	*10*	*1*	*0*	*11*
Biosimilars[1]	**n/a**	**2**	**0**	**2**
Total	**81**	**48**	**7**	**136**

1. Selected disclosed, internal projects. Biosimilar pre-Phase 3 are not disclosed.

INNOVATION

Continuing refinement of R&D portfolio to prioritize high-value transformative medicines

~10%

Total projects in clinical development decreased by **~10%** in Q1 2023 as part of comprehensive portfolio review

136

Current clinical-stage projects in Novartis pipeline

Prioritization based on

- **strategic fit** (within 5 core TAs)
- **asset value**
- **commercial potential**
- **competitive landscape**

Focused portfolio allows

- **greater resource allocation** to priority projects
- **earlier expansion** for high value assets

INNOVATION

Novartis pipeline in Phase 1

20 lead indications

Lead indication

Solid tumors

Code	Name	Mechanism	Indication(s)
AAA603	^{177}Lu-NeoB	Radioligand therapy target GRPR	Multiple solid tumors
AAA817	^{225}Ac-PSMA-617	Radioligand therapy target PSMA	Metastatic castration-resistant prostate cancer
DFF332	DFF332	HIF2A inhibitor	Renal cell carcinoma
IAG933	IAG933	-	Mesothelioma
KAZ954	KAZ954	-	Solid tumors
KFA115	KFA115	Novel immunomodulatory Agent	Solid tumors
MGY825	MGY825	-	NSCLC
NIR178	NIR178	Ad2AR inhibitor	Cancers
NZV930	NZV930, spartalizumab, NIR178	CD73 antagonist	Solid tumors

Immunology

Code	Name	Mechanism	Indication(s)
MHV370	MHV370	-	Systemic lupus erythematosus
NGI226	NGI226	-	Tendinopathy

Neuroscience

Code	Name	Mechanism	Indication(s)
NIO752	NIO752	Tau antagonist	Alzheimer's disease
			Progressive supranuclear palsy

Hematology

Code	Name	Mechanism	Indication(s)
HDM201	HDM201 (combos)	MDM2 inhibitor	Hematological malignancy
JBH492	JBH492	-	Hematological malignancy
MBG453	sabatolimab	TIM3 antagonist	Low risk myelodysplastic syndrome
MIK665	MIK665	MCL1 inhibitor	Hematological malignancies
PIT565	PIT565	-	B-cell malignancies
VAY736	ianalumab + ibrutinib	BAFF-R inhibitor	Hematological malignancy (combo)
			Diffuse large B-cell lymphoma
VOB560	VOB560	-	Cancers
YTB323	rapcabtagene autoleucel	CD19 CAR-T	Adult ALL

Cardiovascular

Code	Name	Mechanism	Indication(s)
XXB750	XXB750	NPR1 agonist	Cardiovascular diseases

Others

Code	Name	Mechanism	Indication(s)
Global Health			
EDI048	EDI048	CpPI(4)K inhibitor	Cryptosporidiosis
EYU688	EYU688	NS4B inhibitor	Dengue
KAF156	ganaplacide	Non-artemisinin plasmodium falciparum inhibitor	Malaria prophylaxis
INE963	INE963	-	Malaria, uncomplicated
Ophthalmology			
MHU650	MHU650	-	Diabetic eye diseases

INNOVATION

Novartis pipeline in Phase 2

23 lead indications

Lead indication

Solid Tumors

Code	Name	Mechanism	Indication(s)
AAA601	Lutathera®	Radioligand therapy target SSTR	GEPNET, pediatrics
			1L ES-SCLC
			Glioblastoma
JDQ443	JDQ443	KRAS inhibitor	NSCLC and CRC (mono and/or combo)
NIS793	niseovkitug	TGFB inhibitor	1L metastatic colorectal cancer
TNO155	TNO155	SHP2 inhibitor	Solid tumors

Immunology

Code	Name	Mechanism	Indication(s)
CFZ533	iscalimab	CD40 inhibitor	Sjögren's
			Hidradenitis suppurativa
CMK389	CMK389	IL-18 inhibitor	Atopic dermatitis
DFV890	DFV890	NLRP3 inhibitor	Knee osteoarthritis
			Familial cold auto-inflammatory syndrome
LNA043	LNA043	ANGPTL3 agonist	Knee osteoarthritis
			Osteoarthritis (combos)
LOU064	remibrutinib	BTK inhibitor	Food allergy
			Hidradenitis suppurativa
			Sjögren's
LRX712	LRX712	-	Osteoarthritis
MAS825	MAS825	-	NLRC4-GOF indications
			Hidradenitis suppurativa
MHV370	MHV370	-	Sjögren's
			Mixed connective tissue disease
QUC398	QUC398	ADAMTS5 inhibitor	Osteoarthritis
VAY736	ianalumab	BAFF-R inhibitor	Autoimmune hepatitis
YTB323	rapcabtagene autoleucel	CD19 CAR-T	Lupus Nephritis

Neuroscience

Code	Name	Mechanism	Indication(s)
BLZ945	sotuletinib	CSF-1R inhibitor	Amyotrophic lateral sclerosis
DLX313[1]	minzasolmin	Alpha-synuclein Inhibitor	Parkinson's disease
MIJ821	onfasprodil	NR2B negative allosteric modulator	Major depressive disorder with acute suicidal ideation or behavior

1. DLX313 is the Novartis compound code for UCB0599.

Hematology

Code	Name	Mechanism	Indication(s)
ABL001	Scemblix®	BCR-ABL inhibitor	Chronic myeloid leukemia, 2L, pediatrics
INC424	Jakavi®	JAK1/2 inhibitor	Acute GVHD, pediatrics
			Chronic GVHD, pediatrics
LNP023	iptacopan	CFB inhibitor	Immune thrombocytopenia
MBG453	sabatolimab	TIM3 antagonist	Unfit acute myeloid leukemia
			Acute myeloid leukemia, maintenance
PHE885	PHE885	BCMA cell therapy	4L multiple myeloma
PKC412	Rydapt®	Multi-targeted kinase inhibitor	Acute myeloid leukemia, pediatrics
YTB323	rapcabtagene autoleucel	CD19 CAR-T	1L high-risk large B-cell lymphoma

Cardiovascular

Code	Name	Mechanism	Indication(s)
CFZ533	iscalimab	CD40 inhibitor	Lupus nephritis
LNP023	iptacopan	CFB inhibitor	Lupus nephritis
MBL949	MBL949	-	Obesity related diseases
TIN816	TIN816	ATP modulator	Acute kidney injury
XXB750	XXB750	NPR1 agonist	Hypertension

Others

Code	Name	Mechanism	Indication(s)
Global Health			
KAE609	cipargamin	PfATP4 inhibitor	Malaria, severe
			Malaria, uncomplicated
KLU156	Ganaplacide + lumefantrine	Non-artemisinin plasmodium falciparum inhibitor	Malaria, uncomplicated
LXE408	LXE408	Proteasome inhibitor	Visceral leishmaniasis
QMF149	Atectura®	Combo	Asthma, pediatrics
SEG101	Adakveo®	P-selectin inhibitor	Sickle cell disease, pediatrics
Respiratory & Allergy			
CMK389	CMK389	IL-18 inhibitor	Pulmonary sarcoidosis
LTP001	LTP001	SMURF1 inhibitor	Pulmonary arterial hypertension
			Idiopathic pulmonary fibrosis
Ophthalmology			
LNP023	iptacopan	CFB inhibitor	iAMD
PPY988[2]	PPY988	Gene therapy - Complement factor I modulation	Geographic atrophy
SAF312	Libvatrep	TRPV1 antagonist	Chronic ocular surface pain

2. Gyroscope acquisition.

INNOVATION

Novartis pipeline in Phase 3

8 lead indications

Lead indication

Solid Tumors

Code	Name	Mechanism	Indication(s)	
AAA617	Pluvicto™	Radioligand therapy target PSMA	mCRPC, pre-taxane	
			Metastatic hormone sensitive prostate cancer (mHSPC)	
AAA601[1]	Lutathera®	Radioligand therapy target SSTR	Gastroenteropancreatic neuroendocrine tumors, 1st line in G2/3 tumors (GEP-NET 1L G3)	
BYL719	Piqray®	PI3Kα inhibitor	Ovarian cancer	
JDQ443	JDQ443	KRAS inhibitor	2/3L Non-small cell lung cancer	
LEE011	Kisqali®	CDK4/6 Inhibitor	HR+/HER2- BC (adj)	
NIS793	niseovkitug	TGFB1 inhibitor	1L Metastatic pancreatic ductal adenocarcinoma	
VDT482	tislelizumab	PD1 inhibitor	1L Nasopharyngeal Carcinoma	Adj/Neo adj. NSCLC
			1L ESCC	1L Gastric cancer
			1L Hepatocellular Carcinoma	Localized ESCC
			1L Urothelial Cell Carcinoma	1L Small Cell Lung Cancer

Immunology

Code	Name	Mechanism	Indication(s)
AIN457	Cosentyx®	IL17A inhibitor	Lupus Nephritis
			Giant cell arteritis
			Polymyalgia rheumatica
			Rotator cuff tendinopathy
IGE025	Xolair®	IgE inhibitor	Food allergy
LOU064	remibrutinib	BTK inhibitor	Chronic spontaneous urticaria
QGE031	ligelizumab	IgE inhibitor	Food allergy
VAY736	ianalumab	BAFF-R inhibitor	Sjögren's
			Lupus Nephritis
			Systemic lupus erythematosus

Neuroscience

Code	Name	Mechanism	Indication(s)
AMG334	Aimovig®	CGRPR antagonist	Migraine, pediatrics
BAF312	Mayzent®	S1P1,5 receptor modulator	Multiple sclerosis, pediatrics
LOU064	remibrutinib	BTK inhibitor	Multiple sclerosis
OAV101	AVXS-101	SMN1 gene replacement therapy	SMA IT administration
OMB157	Kesimpta®	CD20 Antagonist	Multiple sclerosis, pediatrics

Hematology

Code	Name	Mechanism	Indication(s)
ABL001	Scemblix®	BCR-ABL inhibitor	Chronic myeloid leukemia, 1st line
ETB115	Promacta®	Thrombopoietin receptor (TPO-R) agonist	Radiation sickness syndrome
LNP023	iptacopan	CFB inhibitor	Paroxysmal nocturnal hemoglobinuria
			Atypical hemolytic uraemic syndrome
MBG453	sabatolimab	TIM3 antagonist	Myelodysplastic syndrome
VAY736	ianalumab	BAFF-R inhibitor	1L Immune Thrombocytopenia
			2L Immune Thrombocytopenia
			warm Autoimmune Hemolytic Anemia

Cardiovascular

Code	Name	Mechanism	Indication(s)
KJX839	Leqvio®	siRNA (regulation of LDL-C)	CVRR-LDLC
			Primary prevention
			Hyperlipidemia, pediatrics
LNP023	iptacopan	CFB inhibitor	IgA nephropathy
			C3 glomerulopathy
TQJ230	pelacarsen	ASO targeting Lp(a)	Secondary prevention of cardiovascular events in patients with elevated levels of lipoprotein (a) (CVRR-Lp(a))

Others

Code	Name	Mechanism	Indication(s)
Global Health			
COA566	Coartem®	PGH-1 (artemisinin combination therapy)	Malaria, uncomplicated (<5kg patients)
Ophthalmology			
RTH258	Beovu®	VEGF inhibitor	Diabetic retinopathy

Biosimilars

Code	Name	Mechanism	Indication(s)
GP2411	denosumab	anti RANKL mAb	Osteoporosis (same as originator)
SOK583	aflibercept	VEGF inhibitor	Ophthalmology indication (as originator)

1. ^{177}Lu-dotatate in US.

INNOVATION

Novartis pipeline in registration

1 lead indication

Lead indication

Solid Tumors

Code	Name	Mechanism	Indication(s)
VDT482	tislelizumab	PD1 inhibitor	2L ESCC
			Non-small cell lung cancer

Immunology

Code	Name	Mechanism	Indication(s)
AIN457	Cosentyx®	IL17A inhibitor	Hidradenitis suppurativa
			Psoriatic arthritis (IV formulation)
			Axial SpA (IV formulation)
IGE025	Xolair®	IgE inhibitor	Auto-injector

Cardiovascular

Code	Name	Mechanism	Indication(s)
LCZ696	Entresto®	Angiotensin receptor/neprilysin inhibitor	Chronic heart failure, pediatrics[1]

1. Approved in US.

INNOVATION

Novartis submission schedule

New Molecular Entities: Lead and supplementary indications

Cardiovascular | Immunology | Neuroscience | Solid tumors | Hematology | Non-core TA project

Lead

2023	2024	2025	≥2026			
iptacopan LNP023 PNH	**JDQ443** JDQ443 2/3L NSCLC (mono)	**Niseovkitug** NIS793 1L Pancreatic cancer	**^{177}Lu-NeoB** AAA603 Multiple Solid Tumors	**iscalimab** CFZ533 Sjögren's syndrome	**MIJ821** Acute depression	**TNO155** Solid tumors
	remibrutinib LOU064 CSU	**pelacarsen** TQJ230 CVRR-Lp(a)	**ianalumab** VAY736 2L Immune Thrombocytopenia	**ligelizumab** QGE031 Food allergy	**rapcabtagene autoleucel** YTB323 High-risk large B-cell lymphoma	**XXB750** Hypertension
	sabatolimab MBG453 HR-MDS			**LNA043** Knee osteoarthritis		
			cipargamin KAE609 Malaria severe	**libvatrep** SAF312 COSP	**LXE408** Visceral leishmaniasis	**PPY988**[1] Geographic atrophy
			ganaplacide/lumefantrine KLU156 Malaria uncomplicated			

Supplementary

Pluvicto® AAA617 mCRPC, Pre-taxane	**iptacopan** LNP023 C3G		**ianalumab** VAY736 1L Immune Thrombocytopenia	**ianalumab** VAY736 Lupus Nephritis	**rapcabtagene autoleucel** YTB323 Lupus Nephritis	**sabatolimab** MBG453 Unfit AML
tislelizumab VDT482 1L Gastric Cancer	**iptacopan** LNP023 IgAN		**ianalumab** VAY736 wAIHA	**ianalumab** VAY736 SLE	**remibrutinib** LOU064 Multiple sclerosis	**tislelizumab** VDT482 Adj/Neo adj NSCLC
tislelizumab VDT482 1L ESCC	**Pluvicto®** AAA617 mHSPC		**ianalumab** VAY736 AIH	**iptacopan** LNP023 aHUS	**remibrutinib** LOU064 Sjögren's syndrome	**tislelizumab** VDT482 1L Urothelial Cell Carcinoma
tislelizumab VDT482 1L Hepatocellular Carcinoma	**tislelizumab** VDT482 1L Small Cell Lung Cancer		**ianalumab** VAY736 Sjögren's syndrome	**JDQ443** JDQ443 NSCLC (combo)		
tislelizumab VDT482 1L Nasopharyngeal cancer	**tislelizumab** VDT482 Localized ESCC		**cipargamin** KAE609 Malaria uncomplicated			

1. Gyroscope acquisition.

INNOVATION

Novartis submission schedule

Supplementary indications for existing brands

Cardiovascular | Immunology | Neuroscience | Solid tumors | Hematology | Non-core TA project

Existing brands

2023	2024	2025	≥2026			
Kisqali® ribociclib, LEE011 HR+/HER2- BC (adj)	**Jakavi®** ruxolitinib, INC424 Pediatrics Acute GVHD	**Cosentyx®** secukinumab, AIN457 GCA	**Aimovig®** erenumab, AMG334 Pediatric Migraine	**Cosentyx®** secukinumab, AIN457 Polymyalgia rheumatica	**Leqvio®** KJX839 Primary prevention	**Rydapt®** midostaurin, PKC412 Acute myeloid leukemia, pediatrics
Lutathera® ^{177}Lu-oxodotreotide[1] GEP-NET 1L G3	**Jakavi®** ruxolitinib, INC424 Pediatrics Chronic GVHD	**Leqvio®** KJX839 Ped Hyperlipidemia	**Cosentyx®** secukinumab, AIN457 Lupus Nephritis	**Kesimpta® [2]** ofatumumab Multiple sclerosis, pediatrics	**Mayzent® [2]** siponimod, BAF312 Multiple sclerosis, pediatrics	**Scemblix®** ABL001 CML, 2L, pediatrics
Xolair® omalizumab, IGE025 Food allergy	**Scemblix®** ABL001 CML 1L	**Zolgensma®** AVXS-101 OAV101 SMA IT	**Cosentyx®** secukinumab, AIN457 Tendinopathy	**Leqvio®** KJX839 CVRR-LDLC		
Piqray® alpelisib, BYL719 Ovarian cancer						
denosumab BioS GP2411 anti RANKL mAb	**Adakveo** SEG101 Sickle cell disease, pediatrics	**Beovu®** brolucizumab, RTH258 Diabetic retinopathy	**Atectura®** indacaterol + mometasone, QMF149 Asthma, pediatrics			
	aflibercept BioS SOK583 Neovascular age-related macular degeneration	**Promacta®** eltrombopag, ETB115 Radiation sickness syndrome				
	Coartem® artemether + lumefantrine, COA566 Malaria uncompl., formula for <5kg					

1. ^{177}Lu-dotatate in US. 2. Kesimpta and Mayzent: Pediatric study in multiple sclerosis run in conjunction (NEOS).

GROWTH

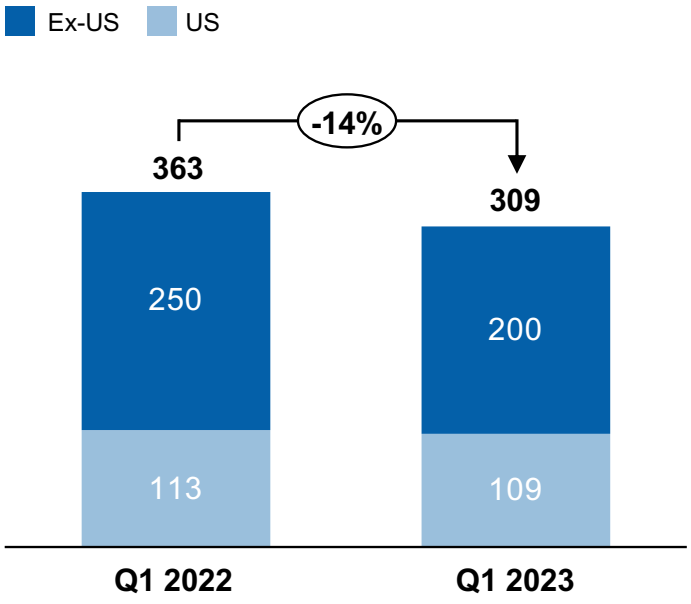
Zolgensma® sales declined mainly due to pricing dynamics

Maintaining leading share in US in <2 years[1]; new data demonstrates durability of effect up to 7.5 years[2,3]

zolgensma®

Sales evolution

USD m, % cc



Q1 sales dynamics driven by one-time reimbursement events in PY and ongoing pricing mix dynamics

Treatment mainly in incident patients; **maintaining >90% share** in US[1]

Continued geographic expansion[2] and access expansion to label

OAV101 **IT development on track** (Ph 3 STEER and STRENGTH trials)

Sustained durability up to 7.5 years (data at MDA)[3]: 25/25 children treated prior to SMA symptom onset achieved walking alone[4,5]

See page 94 for references 1-5. SMA – spinal muscular atrophy. IT – intrathecal. MDA – muscular dystrophy association.

FINANCIAL PROFILE

FY 2023 guidance on other financial KPIs

Barring unforeseen events; (in cc)

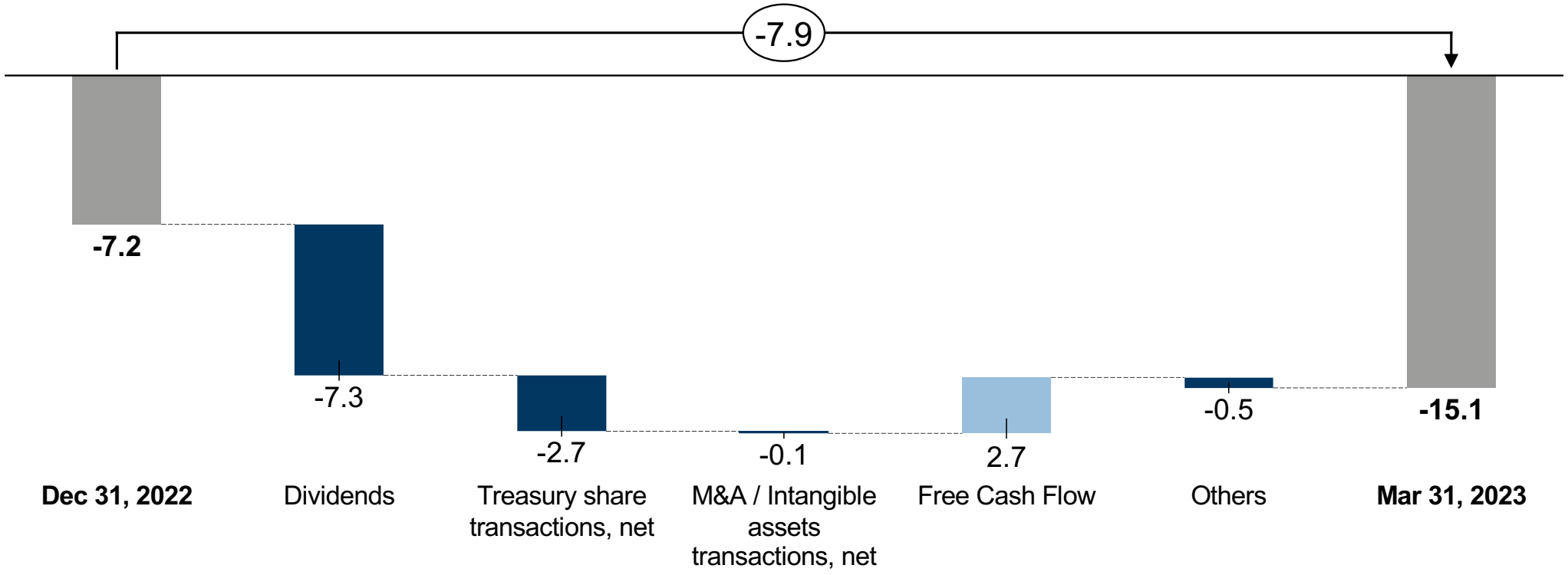
Group | Full year guidance

Core Net Financial Result	Expenses expected to decrease by around 0.1bn vs. 2022 (revised from broadly in line vs. 2022)
Core Tax Rate	Expected to be broadly in line vs. 2022

FINANCIAL PROFILE

Net debt increased by USD 7.9bn mainly due to dividends and share buybacks, partially offset by FCF

USDbn



Clinical Trials Update

Includes selected ongoing or recently concluded global trials of Novartis development programs/products which are in confirmatory development or marketed (typically Phase 2b or later).

For further information on all Novartis clinical trials, please visit: www.novartisclinicaltrials.com

Cardiovascular

iptacopan - CFB inhibitor

NCT04578834 APPLAUSE-IgAN (CLNP023A2301)

Indication	IgA nephropathy
Phase	Phase 3
Patients	450
Primary Outcome Measures	Ratio to baseline in urine protein to creatinine ratio (sampled from 24h urine collection) at 9 months Annualized total estimated Glomerular Filtration Rate (eGFR) slope estimated over 24 months
Arms Intervention	Arm 1 - LNP023 200mg BID Arm 2 - Placebo BID
Target Patients	Primary IgA Nephropathy patients
Readout Milestone(s)	2023 (primary endpoint for US initial submission, 9 months UPCR) 2025 (24 months)
Publication	Perkovic et al. 2021, Nephrology Dialysis Transplantation, Vol. 36, Suppl. 1: Study Design

iptacopan - CFB inhibitor

NCT03955445 (CLNP023B12001B)

Indication	C3 glomerulopathy (C3G)
Phase	Phase 2
Patients	27 patients from ongoing Ph2 (sample size from Ph3 pending HA discussions Q1 2021), total patients for this study will increase
Primary Outcome Measures	Characterize the effect of LNP023 treatment on a composite renal response endpoint at 9 months (1. a stable or improved eGFR and, 2. a reduction in proteinuria and 3. an increase in C3 compared to the CLNP023X2202 baseline visit)
Arms Intervention	Open-label LNP023 200mg bid
Target Patients	Patients with C3 glomerulopathy
Readout Milestone(s)	2025
Publication	Wong et al 2021 Nephrology, Dialysis and Transplantation Vol. 36, Suppl. 1: eGFR trajectory

iptacopan - CFB inhibitor

NCT04817618 APPEAR-C3G (CLNP023B12301)

Indication	C3 glomerulopathy
Phase	Phase 3
Patients	68
Primary Outcome Measures	Log-transformed ratio to baseline in UPCR (sampled from a 24 hour urine collection)
Arms Intervention	Experimental: iptacopan 200mg b.i.d. Placebo Comparator: Placebo to iptacopan 200mg b.i.d.
Target Patients	Patients with native C3G
Readout Milestone(s)	2023
Publication	TBD

Leqvio® - siRNA (regulation of LDL-C)

NCT03705234 ORION-4 (CKJX839B12301)

Indication	Hypercholesterolemia inc. Heterozygous Familial Hypercholesterolaemia (HeFH)
Phase	Phase 3
Patients	15000
Primary Outcome Measures	A composite of major adverse cardiovascular events, defined as: Coronary heart disease (CHD) death; Myocardial infarction; Fatal or non-fatal ischaemic stroke; or Urgent coronary revascularization procedure
Arms Intervention	Arm 1: every 6 month treatment Inclisiran sodium 300mg (given by subcutaneous injection on the day of randomization, at 3 months and then every 6-months) for a planned median duration of about 5 years Arm 2: matching placebo (given bysubcutaneous injection on the day of randomization, at 3 months and then every 6- months) for a planned median duration of about 5 years.
Target Patients	Patient population with mean baseline LDL-C ≥ 100mg/dL
Readout Milestone(s)	2026
Publication	TBD

Leqvio® - siRNA (regulation of LDL-C)

NCT03814187 ORION-8 (CKJX839A12305B)

Indication	Hypercholesterolemia inc. Heterozygous Familial Hypercholesterolaemia (HeFH) and Homozygous Familial Hypercholesterolemia (HoFH)
Phase	Phase 3
Patients	3275
Primary Outcome Measures	Proportion of subjects achieving prespecified low density lipoprotein cholesterol (LDL-C) targets at end of study Safety and tolerability profile of long-term use of inclisiran
Arms Intervention	Inclisiran sodium 300mg on Day 90 and every 180 days for a planned duration of 3 years
Target Patients	Patients with HeFH or pre-existing atherosclerotic cardiovascular disease (ASCVD) on background statin +/- ezetimibe therapy and risk equivalents (patients from ORION 3, 9, 10 & 11 studies)
Readout Milestone(s)	2023
Publication	A pooled safety analysis of inclisiran in 3576 patients with approximately 10,000 person-years of exposure from seven trials; oral presentation; ACC 2-4 Mar-2023 ORION-8 Primary data publication in 2023

Leqvio® - siRNA (regulation of LDL-C)

NCT04652726 ORION-16 (CKJX839C12301)

Indication	Hyperlipidemia, pediatrics
Phase	Phase 3
Patients	141
Primary Outcome Measures	Percentage (%) change in low-density lipoprotein cholesterol (LDL-C) from baseline to Day 330
Arms Intervention	Group 1: Inclisiran sodium 300mg on Days 1, 90, 270, placebo on Day 360, inclisiran sodium 300mg on Days 450 and 630 Group 2: Placebo on Days 1, 90, 270, inclisiran sodium 300mg on Days 360, 450 and 630.
Target Patients	Adolescents (12 to less than 18 years) with heterozygous familial hypercholesterolemia (HeFH) and elevated low density lipoprotein cholesterol (LDL-C)
Readout Milestone(s)	2025
Publication	Design publication (O-16/-13) in Eur. J. Prev. Cardiol. Vol. 29, Feb. 2022 (actual) Presentation at EAS May-2022 on O-13/-16 study design (actual)

Leqvio® - siRNA (regulation of LDL-C)

NCT04659863 ORION-13 (CKJX839C12302)

Indication	Hyperlipidemia, pediatrics
Phase	Phase 3
Patients	13
Primary Outcome Measures	Percentage (%) change in low-density lipoprotein cholesterol (LDL-C) from baseline to day 330
Arms Intervention	Group 1: Inclisiran sodium 300mg on Days 1, 90, 270, placebo on Day 360, inclisiran sodium 300mg on Days 450 and 630. Group 2: Placebo on Days 1, 90, 270, inclisiran sodium 300mg on Days 360, 450 and 630.
Target Patients	Adolescents (12 to less than 18 years) with homozygous familial hypercholesterolemia (HoFH) and elevated low density lipoprotein cholesterol (LDL-C)
Readout Milestone(s)	2025
Publication	Design publication (O-16/-13) in Eur. J. Prev. Cardiol. Vol. 29, Feb. 2022 (actual) Presentation at EAS May-2022 on O-13/-16 study design (actual)

Leqvio® - siRNA (regulation of LDL-C)

NCT05030428 VICTORION-2P (CKJX839B12302)

Indication	Secondary prevention of cardiovascular events in patients with elevated levels of LDL-C
Phase	Phase 3
Patients	15000
Primary Outcome Measures	1. Time to First Occurrence of 3P-MACE (3-Point Major Adverse Cardiovascular Events)
Arms Intervention	Arm 1: Experimental Inclisiran sodium, Subcutaneous injection Arm 2: Placebo Comparator, Placebo Subcutaneous injection
Target Patients	Participants with established cardiovascular disease (CVD)
Readout Milestone(s)	2027
Publication	TBD

Leqvio® - siRNA (regulation of LDL-C)

NCT05739383 VICTORION-1P (CKJX839D12302)

Indication	CVRR (Primary prevention)
Phase	Phase 3
Patients	14000
Primary Outcome Measures	Time to the first occurrence of 4P-MACE 4-Point-Major Adverse Cardiovascular Events (4P-MACE): composite of cardiovascular death, non-fatal myocardial infarction, non-fatal ischemic stroke, and urgent coronary revascularization
Arms Intervention	Arm 1 Experimental: Inclisiran Sodium 300mg, subcutaneous injection in pre-filled syringe Arm 2 Placebo
Target Patients	High-risk primary prevention patients
Readout Milestone(s)	2029
Publication	TBD

pelacarsen - Antisense oligonucleotide (ASO) targeting Lp(a)

NCT04023552 Lp(a)HORIZON (CTQJ230A12301)

Indication	Secondary prevention of cardiovascular events in patients with elevated levels of lipoprotein(a)
Phase	Phase 3
Patients	8323
Primary Outcome Measures	Time to the first occurrence of MACE (cardiovascular death, non-fatal MI, non-fatal stroke and urgent coronary re-vascularization)
Arms Intervention	TQJ230 80 mg injected monthly subcutaneously or matched placebo
Target Patients	Patients with a history of Myocardial infarction or Ischemic Stroke, or a clinically significant symptomatic Peripheral Artery Disease, and Lp(a) ≥ 70 mg/dL
Readout Milestone(s)	2025
Publication	TBD

XXB750 - NPR1 agonist

NCT05562934 (CXXB750B12201)

Indication	Hypertension
Phase	Phase 2b
Patients	170
Primary Outcome Measures	Change from baseline in mean 24hr ambulatory systolic blood pressure at week 12
Arms Intervention	Arm 1 experimental: Dose 1 Arm 2 experimental: Dose 2 Arm 3 experimental: Dose 3 Arm 4 experimental: Dose 4 Arm 5 placebo comparator
Target Patients	Resistant Hypertension Patients
Readout Milestone(s)	2024
Publication	TBD

Immunology

Cosentyx® - IL-17A inhibitor

NCT04181762 SELUNE (CAIN457Q12301)

Indication	Lupus Nephritis
Phase	Phase 3
Patients	460
Primary Outcome Measures	Proportion of subjects achieving protocol-defined CRR
Arms Intervention	Secukinumab 300 mg s.c. Placebo s.c.
Target Patients	Patients with active lupus nephritis (ISN/RPS Class III or IV, with or without co-existing class V features)
Readout Milestone(s)	2025
Publication	TBD

Cosentyx® - IL-17A inhibitor

NCT04930094 GCAPTAIN (CAIN457R12301)

Indication	Giant cell arteritis
Phase	Phase 3
Patients	348
Primary Outcome Measures	Number of participants with sustained remission
Arms Intervention	Experimental: Secukinumab 300 mg Placebo Comparator: Placebo
Target Patients	Patients with Giant Cell Arteritis (GCA)
Readout Milestone(s)	Primary 2025 Final 2026
Publication	TBD

Cosentyx® - IL-17A inhibitor

NCT05722522 (CAIN457O12301)

Indication	Rotator cuff tendinopathy
Phase	Phase 3
Patients	234
Primary Outcome Measures	Change from BSL in in the Western Ontario Rotator Cuff Index (WORC) Physical Symptom Domain (PSD) score [Time Frame: At Week 16]: - Improving physical shoulder symptoms in participants with moderate to severe RCT at Week 16
Arms Intervention	Arm 1: Secukinumab 2 X 150 mg / 1 mL, subcutaneous (s.c.) injection, randomized in a 1:1 ratio Arm 2: Placebo 2X 1 mL, subcutaneous (s.c.) injection, randomized in a 1:1 ratio
Target Patients	Patients with moderate-severe Rotator Cuff Tendinopathy
Readout Milestone(s)	2025
Publication	TBD

Cosentyx® - IL-17A inhibitor

NCT05758415 (CAIN457O12302)

Indication	Rotator cuff tendinopathy
Phase	Phase 3
Patients	234
Primary Outcome Measures	Change from BSL in in the Western Ontario Rotator Cuff Index (WORC) Physical Symptom Domain (PSD) score [Time Frame: At Week 16]: - Change in physical shoulder symptoms in participants with moderate to severe RCT at Week 16
Arms Intervention	Arm 1 experimental: Secukinumab 2 X 150 mg / 1 mL, subcutaneous (s.c.) injection, randomized in a 1:1 ratio Arm 2 placebo: 2 X 1 mL, subcutaneous (s.c.) injection, randomized in a 1:1 ratio
Target Patients	Patients with moderate-severe Rotator Cuff Tendinopathy
Readout Milestone(s)	2025
Publication	TBD

Cosentyx® - IL-17A inhibitor

NCT05767034 REPLENISH (CAIN457C22301)

Indication	Polymyalgia rheumatica
Phase	Phase 3
Patients	360
Primary Outcome Measures	Proportion of participants achieving sustained remission
Arms Intervention	Arm 1 Experimental: Secukinumab 300 mg, randomized in 1:1:1 ratio every 4 weeks Arm 2 Experimental: Secukinumab 150 mg, randomized in 1:1:1 ratio every 4 weeks Arm 3 Placebo : randomized in 1:1:1 ratio every 4 weeks
Target Patients	Adult patients with PMR who have recently relapsed
Readout Milestone(s)	2025
Publication	TBD

ianalumab - BAFF-R inhibitor

NCT03217422 AMBER (CVAY736B2201)

Indication	Autoimmune hepatitis
Phase	Phase 2
Patients	65
Primary Outcome Measures	Alanine aminotransferase (ALT) normalization
Arms Intervention	VAY736 Placebo control with conversion to active VAY736
Target Patients	Autoimmune hepatitis patients with incomplete response or intolerant to standard treatment of care
Readout Milestone(s)	2024
Publication	TBD

ianalumab - BAFF-R inhibitor

NCT05126277 SIRIUS-LN (CVAY736K12301)

Indication	Lupus Nephritis
Phase	Phase 3
Patients	420
Primary Outcome Measures	Frequency and percentage of participants achieving complete renal response (CRR) [Time Frame: week 72]
Arms Intervention	Arm 1: Experimental - ianalumab s.c. q4w in addition to standard of care (SoC) Arm 2: Experiemental - ianalumab s.c. q12w in addition to SoC Arm 3: Placebo comparator - Placebo s.c. q4w in addition to SoC
Target Patients	Patients with active Lupus Nephritis
Readout Milestone(s)	Primary 2027
Publication	TBD

ianalumab - BAFF-R inhibitor

NCT05349214 NEPTUNUS-2 (CVAY736A2302)

Indication	Sjögren's syndrome
Phase	Phase 3
Patients	489
Primary Outcome Measures	Change from baseline in EULAR Sjögren Syndrome Disease Activity Index (ESSDAI) score at Week 48 as compared to placebo
Arms Intervention	Arm 1: Experimental - ianalumab exposure level 1 Arm 2: Experimental - ianalumab exposure level 2 Arm 3: Placebo comparator
Target Patients	Patients with active Sjogren's syndrome
Readout Milestone(s)	Primary 2026
Publication	TBD

ianalumab - BAFF-R inhibitor

NCT05350072 NEPTUNUS-1 (CVAY736A2301)

Indication	Sjögren's syndrome
Phase	Phase 3
Patients	285
Primary Outcome Measures	Change from baseline in EULAR Sjögren Syndrome Disease Activity Index (ESSDAI) score at Week 48 as compared to placebo
Arms Intervention	Arm 1: Experimental - ianalumab Arm 2: Placebo comparator
Target Patients	Patients with active Sjogren's syndrome
Readout Milestone(s)	Primary 2026
Publication	TBD

ianalumab - BAFF-R inhibitor

NCT05639114 SIRIUS-SLE 1 (CVAY736F12301)

Indication	Systemic lupus erythematosus
Phase	Phase 3
Patients	406
Primary Outcome Measures	Proportion of participants on monthly ianalumab achieving Systemic Lupus Erythematosus Responder Index -4 (SRI-4) [Time Frame: Week 60]
Arms Intervention	Experimental: Ianalumab s.c. monthly Experimental: Ianalumab s.c. quarterly Placebo Comparator: Placebo s.c. monthly
Target Patients	Patients with active systemic lupus erythematosus (SLE)
Readout Milestone(s)	2027
Publication	TBD

ianalumab - BAFF-R inhibitor

NCT05624749 SIRIUS-SLE 2 (CVAY736F12302)

Indication	Systemic lupus erythematosus
Phase	Phase 3
Patients	280
Primary Outcome Measures	Proportion of participants achieving Systemic Lupus Erythematosus Responder Index -4 (SRI-4) [Time Frame: Week 60]
Arms Intervention	Experimental: ianalumab s.c. monthly Placebo Comparator: placebo s.c. monthly
Target Patients	Patients with active systemic lupus erythematosus (SLE)
Readout Milestone(s)	2027
Publication	TBD

ligelizumab - IgE Inhibitor

NCT04984876 (CQGE031G12301)

Indication	Food allergy
Phase	Phase 3
Patients	486
Primary Outcome Measures	1. Proportion of participants who can tolerate a single dose of ≥ 600 mg (1044 mg cumulative tolerated dose) of peanut protein without dose-limiting symptoms at Week 12
Arms Intervention	Arm 1: ligelizumab 240 mg subcutaneous injection for 52 weeks Arm 2: ligelizumab 120 mg subcutaneous injection for 52 weeks Arm 3: Placebo subcutaneous injection for first 8 weeks and ligelizumab 120 mg subcutaneous injection for 44 weeks Arm 4: Placebo 16 weeks and ligelizumab 120 mg/240 mg subcutaneous injection for 36 weeks Arm 5: Placebo subcutaneous injection for first 8 weeks and ligelizumab 240 mg subcutaneous injection for 44 weeks
Target Patients	Participants with a medically confirmed diagnosis of IgE-mediated peanut allergy
Readout Milestone(s)	2025
Publication	TBD

LNA043 - ANGPTL3 agonist

NCT04864392 ONWARDS (CLNA043A12202)

Indication	Knee osteoarthritis
Phase	Phase 2
Patients	550
Primary Outcome Measures	Change from baseline in the cartilage thickness of the medial compartment of the knee as assessed by imaging
Arms Intervention	LNA043 injection to the knee with dosing regimen A LNA043 injection to the knee with dosing regimen B LNA043 injection to the knee with dosing regimen C LNA043 injection to the knee with dosing regimen D Placebo injection to the knee
Target Patients	Patients with Symptomatic knee osteoarthritis
Readout Milestone(s)	Primary 2024
Publication	TBD

remibrutinib - BTK inhibitor

NCT05030311 REMIX-1 (CLOU064A2301)

Indication	Chronic spontaneous urticaria
Phase	Phase 3
Patients	450
Primary Outcome Measures	Change from baseline in UAS7 (Scenario 1 with UAS7 as primary efficacy endpoint)
Arms Intervention	Arm 1: LOU064 (blinded) LOU064 (blinded) taken orally for 24 weeks, followed by LOU064 (open-label) taken orally open label for 28 weeks. Randomized in a 2:1 ratio (arm 1:arm 2) Arm 2: LOU064 placebo (blinded) LOU064 placebo (blinded) taken orally for 24 weeks, followed by LOU064 (open-label) taken orally for 28 weeks. Randomized in a 2:1 ratio (arm 1:arm 2)
Target Patients	Adult Chronic Spontaneous Urticaria (CSU) patients inadequately controlled by H1-antihistamines
Readout Milestone(s)	2024 (Final)
Publication	TBD

remibrutinib - BTK inhibitor

NCT05032157 REMIX-2 (CLOU064A2302)

Indication	Chronic spontaneous urticaria
Phase	Phase 3
Patients	450
Primary Outcome Measures	1. Change from baseline in UAS7 (Scenario 1 with UAS7 as primary efficacy endpoint) 2. Absolute change in ISS7 an absolute change in HSS7 (Scenario 2 with ISS7 and HSS7 as co-primary efficacy endpoints)
Arms Intervention	Arm 1: LOU064 (blinded) LOU064A (blinded) taken orally b.i.d. for 24 weeks, followed by LOU064 (open-label) taken orally open label for 28 weeks Arm 2: LOU064 placebo (blinded) LOU064A placebo (blinded) taken orally for 24 weeks, followed by LOU064 (open-label) taken orally open label for 28 weeks Eligible participants randomized to the treatment arms in a 2:1 ratio (arm 1: arm 2)
Target Patients	Adult participants suffering from chronic spontaneous urticaria (CSU) inadequately controlled by H1-antihistamines in comparison to placebo
Readout Milestone(s)	2024 (Final)
Publication	TBD

Neuroscience

Mayzent® - S1P1,5 receptor modulator

NCT04926818 NEOS (CBAF312D2301)

Indication	Multiple sclerosis, pediatrics
Phase	Phase 3
Patients	180
Primary Outcome Measures	Annualized relapse rate (ARR) in target pediatric participants
Arms Intervention	Arm 1: Experimental ofatumumab - 20 mg injection/ placebo Arm 2: Experimental siponimod - 0.5 mg, 1 mg or 2 mg/ placebo Arm 3: Active Comparator fingolimod - 0.5 mg or 0.25 mg/ placebo
Target Patients	Children/adolescent patients aged 10-17 years old with Multiple Sclerosis (MS). The targeted enrollment is 180 participants with multiple sclerosis which will include at least 5 participants with body weight (BW) ≤40 kg and at least 5 participants with age 10 to 12 years in each of the ofatumumab and siponimod arms. There is a minimum 6 month follow up period for all participants (core and extension). Total duration of the study could be up to 7 years.
Readout Milestone(s)	2026
Publication	TBD

MIJ821 - NR2B negative allosteric modulator (NAM)

NCT04722666 (CMIJ821A12201)

Indication	Major depressiv disorder with acute suicidal ideation or behavior
Phase	Phase 2
Patients	195
Primary Outcome Measures	Change from baseline to 24 hours in the total score of the Montgomery Åsberg Depression Rating Scale (MADRS)
Arms Intervention	MIJ821 (mg/kg) very low dose for 40 minutes IV infusion on Day 1, Day 15 and Day 29 MIJ821 (mg/kg) low dose for 40 minutes IV infusion on Day 1, Day 15 and Day 29 MIJ821 (mg/kg) high dose for 40 minutes IV infusion on Day 1, Day 15 and Day 29 MIJ821 (mg/kg) very high dose for 40 minutes IV infusion on Day 1, Day 15 and Day 29 Placebo 40 minutes IV infusion of 0.9% sodium chloride on Day 1, Day 15 and Day 29 MIJ821 (mg/kg) high dose for 40 minutes IV infusion on Day 1 followed by Placebo 40 minutes IV infusion of 0.9% sodium chloride on Day 15 and Day 29 MIJ821 (mg/kg) very high dose for 40 minutes IV infusion on Day 1 followed by Placebo 40 minutes IV infusion of 0.9% sodium chloride on Day 15 and Day 29
Target Patients	Participants who have suicidal ideation with intent
Readout Milestone(s)	2023 (interim)
Publication	TBD

remibrutinib - BTK inhibitor

NCT05147220 REMODEL-1 (CLOU064C12301)

Indication	Multiple sclerosis
Phase	Phase 3
Patients	800
Primary Outcome Measures	Annualized relapse rate (ARR) of confirmed relapses [Core Part]. ARR is the average number of confirmed MS relapses in a year
Arms Intervention	Arm 1: Experimental; Remibrutinib - Core (Remibrutinib tablet and matching placebo of teriflunomide capsule) Arm 2: Active Comparator; Teriflunomide - Core (Teriflunomide capsule and matching placebo remibrutinib tablet) Arm 3: Experimental; Remibrutinib - Extension (Participants on remibrutinib in Core will continue on remibrutinib tablet) Arm 4: Experimental; Remibrutinib - Extension (on teriflunomide in Core) (Participants on teriflunomide in Core will switch to remibrutinib tablet)
Target Patients	Patients with relapsing Multiple Sclerosis
Readout Milestone(s)	Estimated primary completion 2026
Publication	TBD

remibrutinib - BTK inhibitor

NCT05156281 REMODEL-2 (CLOU064C12302)

Indication	Multiple sclerosis
Phase	Phase 3
Patients	800
Primary Outcome Measures	Annualized relapse rate (ARR) of confirmed relapses
Arms Intervention	Arm 1: Experimental; Remibrutinib – Core Remibrutinib tablet and matching placebo of teriflunomide capsule Arm 2: Active Comparator; Teriflunomide – Core Teriflunomide capsule and matching placebo remibrutinib tablet Arm 3: Experimental: Remibrutinib – Extension Participants on remibrutinib in Core will continue on remibrutinib tablet Arm 4: Experimental: Remibrutinib - Extension (on teriflunomide in Core) Participants on teriflunomide in Core will switch to remibrutinib tablet
Target Patients	Patients with relapsing Multiple Sclerosis
Readout Milestone(s)	Estimated primary completion 2026
Publication	TBD

Zolgensma® - SMN1 gene replacement therapy

NCT05089656 STEER (COAV101B12301)

Indication	Spinal muscular atrophy (IT administration)
Phase	Phase 3
Patients	125
Primary Outcome Measures	1. Change from baseline in Hammersmith functional motor scale - Expanded (HFMSE) total score at the end of follow-up period 1 in treated patients compared to sham controls in the ≥ 2 to < 18 years age group
Arms Intervention	Arm 1: Experimental OAV101. Administered as a single, one-time intrathecal dose Arm 2: Sham Comparator: Sham control. A skin prick in the lumbar region without any medication.
Target Patients	Patients Type 2 Spinal Muscular Atrophy (SMA) who are ≥ 2 to < 18 years of age, treatment naive, sitting, and never ambulatory
Readout Milestone(s)	2024
Publication	TBD

Zolgensma® - SMN1 gene replacement therapy

NCT05386680 STRENGTH (COAV101B12302)

Indication	Spinal muscular atrophy (IT administration)
Phase	Phase 3B
Patients	28
Primary Outcome Measures	Number and percentage of participants reporting AEs, related AEs, SAEs, and AESIs [Time Frame: 52 weeks]
Arms Intervention	Experimental: OAV-101 Single intrathecal administration of OAV101 at a dose of 1.2 x 10^14 vector genomes
Target Patients	Participants with SMA who discontinued treatment With Nusinersen or Risdiplam (STRENGTH)
Readout Milestone(s)	2024
Publication	TBD

Oncology

ianalumab - BAFF-R inhibitor

NCT05653349 VAYHIT1 (CVAY736I12301)

Indication	1L Immune Thrombocytopenia
Phase	Phase 3
Patients	225
Primary Outcome Measures	Time from randomization to treatment failure (TTF)
Arms Intervention	Arm 1: Experimental: Ianalumab Lower dose administered intravenously with corticosteroids oral or parentally (if clinically justified) Arm 2: Ianalumab Higher dose administered intravenously with corticosteroids oral or parentally (if clinically justified) Arm 3: Placebo Comparator administered intravenously with corticosteroids oral or parentally (if clinically justified)
Target Patients	Adult patients with primary ITP
Readout Milestone(s)	2025
Publication	TBD

ianalumab - BAFF-R inhibitor

NCT05653219 VAYHIT2 (CVAY736Q12301)

Indication	2L Immune Thrombocytopenia
Phase	Phase 3
Patients	150
Primary Outcome Measures	Time from randomization to treatment failure (TTF)
Arms Intervention	Arm 1: Experimental: eltrombopag and ianalumab lower dose Arm 2: Experimental: eltrombopag and ianalumab higher dose Arm 3: eltrombopag and placebo
Target Patients	Primary ITP patients who failed steroids
Readout Milestone(s)	2025
Publication	TBD

ianalumab - BAFF-R inhibitor

NCT05648968 VAYHIA (CVAY736O12301)

Indication	Warm autoimmune hemolytic anemia
Phase	Phase 3
Patients	90
Primary Outcome Measures	Binary variable indicating whether a patient achieves a durable response Durable response: hemoglobin level ≥10 g/dL and ≥2 g/dL increase from baseline, for a period of at least eight consecutive weeks between W9 and W25, in the absence of rescue medication or prohibited treatment
Arms Intervention	Arm 1: experimental Ianalumab low dose (intravenously) Arm 2: experimental Ianalumab high dose (intravenously) Arm 3: placebo Comparatorn (intravenously)
Target Patients	Previously treated patients with warm Autoimmune Hemolytic Anemia
Readout Milestone(s)	2026
Publication	TBD

iptacopan - CFB inhibitor

NCT04889430 APPELHUS (CLNP023F12301)

Indication	Atypical haemolytic uraemic syndrome
Phase	Phase 3
Patients	50
Primary Outcome Measures	Percentage of participants with complete TMA response without the use of PE/PI and anti-C5 antibody
Arms Intervention	Single arm open-label with 50 adult patients receiving 200mg oral twice daily doses of iptacopan
Target Patients	Adult patients with aHUS who are treatment naive to complement inhibitor therapy (including anti-C5 antibody)
Readout Milestone(s)	2025
Publication	TBD

Jakavi® - JAK1/2 inhibitor

NCT03491215 REACH4 (CINC424F12201)

Indication	Acute graft versus host disease
Phase	Phase 2
Patients	45
Primary Outcome Measures	Measurement of PK parameters Overall Response Rate (ORR)
Arms Intervention	Ruxolitinib
Target Patients	Pediatric patients with grade II-IV acute graft vs. host disease after allogeneic hematopoietic stem cell transplantation
Readout Milestone(s)	2023
Publication	TBD

Jakavi® - JAK1/2 inhibitor

NCT03774082 REACH5 (CINC424G12201)

Indication	Chronic graft versus host disease
Phase	Phase 2
Patients	45
Primary Outcome Measures	Overall Response Rate (ORR)
Arms Intervention	Ruxolitinib 5mg tablets / pediatric formulation
Target Patients	Pediatric subjects with moderate and severe chronic Graft vs. Host disease after allogeneic stem cell transplantation
Readout Milestone(s)	2023
Publication	TBD

JDQ443 - KRAS inhibitor

NCT05132075 KontRASt-02 (CJDQ443B12301)

Indication	Non-small cell lung cancer, 2/3L
Phase	Phase 3
Patients	360
Primary Outcome Measures	Progression free survival (PFS)
Arms Intervention	Arm 1 Experimental: JDQ443 Arm 2 Active Comparator: Participant will be treated with docetaxel following local guidelines as per standard of care and product labels
Target Patients	Patients with advanced non-small cell lung cancer (NSCLC) harboring a KRAS G12C mutation who have been previously treated with a platinum-based chemotherapy and immune checkpoint inhibitor therapy either in sequence or in combination.
Readout Milestone(s)	2024
Publication	NA

Kisqali® - CDK4 inhibitor

NCT03701334 NATALEE (CLEE011O12301C)

Indication	Adjuvant treatment of hormone receptor (HR)-positive, HER2-negative, early breast cancer (EBC)
Phase	Phase 3
Patients	5101
Primary Outcome Measures	Invasive Disease-Free Survival for using STEEP criteria (Standardized Definitions for Efficacy End Points in adjuvant breast cancer trials)
Arms Intervention	Ribociclib + endocrine therapy Endocrine therapy
Target Patients	Pre and postmenopausal women and men with HR-positive, HER2-negative EBC, after adequate surgical resection, who are eligible for adjuvant endocrine therapy
Readout Milestone(s)	2023 (actual)
Publication	TBD

niseovkitug - TGF-beta 1 inhibitor

NCT04935359 daNIS-2 (CNIS793B12301)

Indication	1L metastatic pancreatic ductal Adenocarcinoma
Phase	Phase 3
Patients	501
Primary Outcome Measures	Safety run-in part: Percentage of participants with dose limiting toxicities (DLTs) during the first cycle (4 weeks) of treatment Randomized part: Overall survival (OS)
Arms Intervention	Safety run-in part: NIS793+gemcitabine+nab-paclitaxel Randomized portion of the study: Arm 1: NIS793+gemcitabine+nab-paclitaxel Arm 2: placebo+gemcitabine+nab-paclitaxel
Target Patients	Patients with Metastatic Pancreatic Ductal Adenocarcinoma (mPDAC), first line treatment
Readout Milestone(s)	Primary: 2024
Publication	NA

Piqray® - PI3K-alpha inhibitor

NCT04729387 EPIK-O (CBYL719K12301)

Indication	Ovarian Cancer
Phase	Phase 3
Patients	358
Primary Outcome Measures	Progression Free Survival (PFS) based on Blinded Independent Review Committee (BIRC) assessment using RECIST 1.1 criteria
Arms Intervention	Arm 1 Experimental: Alpelisib+olaparib: Alpelisib 200 mg orally once daily and olaparib 200 mg orally twice daily on a continuous dosing schedule Arm 2 Active Comparator: Paclitaxel or PLD. Investigator's choice of one of 2 single agent cytotoxic chemotherapies: Paclitaxel 80 mg/m2 intravenously weekly or Pegylated liposomal Doxorubicin (PLD) 40-50 mg/m2 (physician discretion) intravenously every 28 days.
Target Patients	Patients with platinum resistant or refractory high-grade serous ovarian cancer, with no germline BRCA mutation detected
Readout Milestone(s)	2023
Publication	TBD

Pluvicto® - Radioligand therapy target PSMA

NCT04689828 PSMAfore (CAAA617B12302)

Indication	Metastatic castration-resistant prostate cancer, pre-taxane
Phase	Phase 3
Patients	450
Primary Outcome Measures	Radiographic Progression Free Survival (rPFS)
Arms Intervention	Arm 1: Participants will receive 7.4 GBq (200 mCi) +/- 10% ^{177}Lu-PSMA-617 once every 6 weeks for 6 cycles. Best supportive care, including ADT may be used Arm 2: For participants randomized to the ARDT arm, the change of ARDT treatment will be administered per the physician's orders. Best supportive care, including ADT may be used
Target Patients	mCRPC patients that were previously treated with an alternate ARDT and not exposed to a taxane-containing regimen in the CRPC or mHSPC settings
Readout Milestone(s)	Primary Analysis: 2022 (actual) Final Analysis: 2025
Publication	TBD

Pluvicto® - Radioligand therapy target PSMA

NCT04720157 PSMAddition (CAAA617C12301)

Indication	Metastatic hormone sensitive prostate cancer
Phase	Phase 3
Patients	1126
Primary Outcome Measures	Radiographic Progression Free Survival (rPFS)
Arms Intervention	Arm 1: ^{177}Lu-PSMA-617 Participant will receive 7.4 GBq (+/- 10%) ^{177}Lu-PSMA-617, once every 6 weeks for a planned 6 cycles, in addition to the Standard of Care (SOC); ARDT +ADT is considered as SOC and treatment will be administered per the physician's order Arm 2: For participants randomized to Standard of Care arm, ARDT +ADT is considered as SOC and treatment will be administered per the physician's order
Target Patients	Patients with metastatic Hormone Sensitive Prostate Cancer (mHSPC)
Readout Milestone(s)	Primary Analysis: 2024
Publication	TBD

Rydapt® - Multi-targeted kinase inhibitor

NCT03591510 (CPKC412A2218)

Indication	Acute myeloid leukemia, pediatrics
Phase	Phase 2
Patients	20
Primary Outcome Measures	Occurrence of dose limiting toxicities Safety and Tolerability
Arms Intervention	Chemotherapy followed by Midostaurin
Target Patients	Newly diagnosed pediatric patients with FLT3 mutated acute myeloid leukemia (AML)
Readout Milestone(s)	2026
Publication	TBD

sabatolimab - TIM3 antagonist

NCT04150029 STIMULUS-AML1 (CMBG453C12201)

Indication	Unfit acute myeloid leukaemia
Phase	Phase 2
Patients	86
Primary Outcome Measures	Incidence of dose limiting toxicities (Safety run-in patients only) Percentage of subjects achieving complete remission (CR)
Arms Intervention	Single arm safety and efficacy study of sabatolimab in combination with azacitidine and venetoclax
Target Patients	Newly diagnosed adult AML patients who are not suitable for treatment with intensive chemotherapy
Readout Milestone(s)	2023
Publication	TBD

sabatolimab - TIM3 antagonist

NCT04266301 STIMULUS-MDS2 (CMBG453B12301)

Indication	Myelodysplastic syndrome
Phase	Phase 3
Patients	500
Primary Outcome Measures	Overall survival
Arms Intervention	Sabatolimab 800 mg + azacitidine 75 mg/m2 Sabatolimab 800 mg + azacitidine 75 mg/m2 + placebo
Target Patients	Patients with intermediate, high or very high risk Myelodysplastic Syndrome (MDS) as Per IPSS-R, or Chronic Myelomonocytic Leukemia-2 (CMML-2)
Readout Milestone(s)	2024
Publication	TBD

Scemblix® - BCR-ABL inhibitor

NCT04971226 ASC4FIRST (CABL001J12301)

Indication	Chronic myeloid leukemia, 1st line
Phase	Phase 3
Patients	402
Primary Outcome Measures	Major Molecular Response (MMR) at week 48
Arms Intervention	Arm 1: asciminib 80 mg QD Arm 2: Investigator selected TKI including one of the below treatments: - Imatinib 400 mg QD - Nilotinib 300 mg BID - Dasatinib 100 mg QD - Bosutinib 400 mg QD
Target Patients	Patients with newly diagnosed philadelphia chromosome positive chronic myelogenous leukemia in chronic phase
Readout Milestone(s)	2024
Publication	TBD

TNO155 - SHP2 inhibitor

NCT03114319 (CTNO155X2101)

Indication	Solid tumors (single agent)
Phase	Phase 1
Patients	255
Primary Outcome Measures	Number of participants with adverse events Number of participants with dose limiting toxicities
Arms Intervention	Drug: TNO155 Drug: TNO155 in combination with EGF816 (nazartinib)
Target Patients	Adult patients with advanced solid tumors in selected indications
Readout Milestone(s)	2024
Publication	TBD

Other

Ophthalmology

Beovu® - VEGF Inhibitor

NCT04278417 CONDOR (CRTH258D2301)

Indication	Diabetic retinopathy
Phase	Phase 3
Patients	694
Primary Outcome Measures	Change from Baseline in BCVA
Arms Intervention	Arm 1: RTH258 (brolucizumab) 6 mg/50uL Arm 2: Panretinal photocoagulation laser initial treatment followed with additional PRP treatment as needed
Target Patients	Patients with proliferative diabetic retinopathy
Readout Milestone(s)	2024
Publication	TBD

libvatrep - TRPV1 antagonist

NCT04630158 SAHARA (CSAF312B12201)

Indication	Chronic ocular surface pain
Phase	Phase 2
Patients	150
Primary Outcome Measures	Change in mean pain severity Visual Analog Scale
Arms Intervention	Placebo Comparator: SAF312 Placebo. Randomized to a 1:1:1 topical eye drops, twice daily Experimental: SAF312 dose 1. Randomized to a 1:1:1 topical eye drops, twice daily Experimental: SAF312 dose 2. Randomized to a 1:1:1 topical eye drops, twice daily
Target Patients	Subjects with CICP persisting at least for 4 months after refractive surgery and chronicity confirmed during the observational period.
Readout Milestone(s)	2023
Publication	2023

Global Health

Adakveo® - P-selectin inhibitor

NCT03474965 SOLACE-Kids (CSEG101B2201)

Indication	Sickle cell disease, pediatrics
Phase	Phase 2
Patients	100
Primary Outcome Measures	PK/PD and safety of SEG101 at 5 mg/kg
Arms Intervention	SEG101 (crizanlizumab) at a dose of 5 mg/kg by IV infusion ± Hydroxyurea/Hydroxycarbamide
Target Patients	Pediatric SCD patients with VOC
Readout Milestone(s)	H2-2021 (pediatric patients ≥12 year old) 2024 (pediatric patients <12 year old)
Publication	1. Matthew M. Heeney, David C. Rees, Mariane de Montalembert, Isaac Odame, R. Clark Brown, Yasser Wali, Thu Thuy Nguyen, Du Lam, Raquel Merino Herranz, Julie Kanter; Study Design and Initial Baseline Characteristics in Solace-Kids: Crizanlizumab in Pediatric Patients with Sickle Cell Disease. Blood 2020; 136 (Supplement 1): 22–24. doi: https://doi.org/10.1182/blood-2020-137081 2. Matthew M. Heeney, David C. Rees, Mariane De Montalembert, Isaac Odame, R. Clark Clark Brown, Yasser Wali, Thu Thuy Nguyen, Du Lam, Nadege Pfender, Julie Kanter; Initial Safety and Efficacy Results from the Phase II, Multicenter, Open-Label Solace-Kids Trial of Crizanlizumab in Adolescents with Sickle Cell Disease (SCD). Blood 2021; 138 (Supplement 1): 12. doi: https://doi.org/10.1182/blood-2021-144730

cipargamin - PfATP4 inhibitor

NCT04675931 KARISMA (CKAE609B12201)

Indication	Malaria severe
Phase	Phase 2
Patients	252
Primary Outcome Measures	Percentage of participants achieving at least 90% reduction in Plasmodium falciparum (P. falciparum) at 12 hours [Time Frame: Day 1 (12 Hours)]
Arms Intervention	Arm 1: experimental, IV KAE609 Dose regimen 1 Arm 2: experimental, IV KAE609 Dose regimen 2 Arm 3: experimental, IV KAE609 Dose regimen 3 Arm 4: active comparator, IV Artesunate Arm 5: Coartem, Standard of care
Target Patients	Patients with Malaria, severe
Readout Milestone(s)	2024
Publication	TBD

Coartem® - PGH-1 (artemisinin combination therapy)

NCT04300309 CALINA (CCOA566B2307)

Indication	Malaria, uncomplicated (<5kg patients)
Phase	Phase 3
Patients	44
Primary Outcome Measures	Artemether Cmax
Arms Intervention	Experimental: artemether lumefantrine (2.5 mg:30 mg) artemether lumefantrine (2.5 mg:30 mg) bid over 3 days, from 1-4 tablets per dose
Target Patients	Infants and Neonates <5 kg body weight with acute uncomplicated plasmodium falciparum malaria
Readout Milestone(s)	Primary outcome measure: 2023
Publication	TBD

ganaplacide - Non-artemisinin plasmodium falciparum inhibitor

NCT04546633 KALUMI (CKAF156A2203)

Indication	Malaria, uncomplicated
Phase	Phase 2
Patients	292
Primary Outcome Measures	PCR-corrected and uncorrected Adequate Clinical and Parasitological Response (ACPR)
Arms Intervention	KAF156 and LUM-SDF QD (once daily) for 2 days in fasted condition KAF156 and LUM-SDF QD (once daily) for 2 days in fed condition
Target Patients	Malaria patients 6 months to < 18 years old
Readout Milestone(s)	2023
Publication	TBD

Biosimilars

aflibercept - VEGF inhibitor

NCT04864834 Mylight (CSOK583A12301)

Indication	Ophthalmology indication (as originator)
Phase	Phase 3
Patients	460
Primary Outcome Measures	Best-corrected visual acuity (BCVA) will be assessed using the ETDRS testing charts at an initial distance of 4 meters. The change from baseline in BCVA in letters is defined as difference between BCVA score between week 8 and baseline
Arms Intervention	Arm 1 Biological: SOK583A1 (40 mg/mL) Arm 2 Biological: Eylea EU (40 mg/mL)
Target Patients	Patients with neovascular age-related macular degeneration
Readout Milestone(s)	2023
Publication	tbd

Abbreviations

AI	Auto-injector
AIH	Autoimmune hepatitis
aHUS	atypical Hemolytic Uremic Syndrome
ALL	Acute lymphoblastic leukemia
ALS	Amyotrophic lateral sclerosis
AML	Acute myeloid leukemia
BC	Breast cancer
C3G	C3 glomerulopathy
CART	Chimeric androgen receptor T
CLL	Chronic lymphocytic leukemia
CML	Chronic myeloid leukemia
CRC	Colorectal cancer
COPD	Chronic obstructive pulmonary disease
COSP	Chronic ocular surface pain
CSU	Chronic spontaneous urticaria
CVRR-Lp(a)	Secondary prevention of cardiovascular events in patients with elevated levels of lipoprotein (a)
CVRR-LDLC	Secondary prevention of cardiovascular events in patients with elevated levels of LDLC
DME	Diabetic macular edema
DLBCL	Diffuse large B-cell lymphoma refractory
ESCC	Esophageal squamous-cell carcinoma
FL	Follicular lymphoma
GCA	Giant cell arteritis
GVHD	Graft-versus-host disease
GRPR	Gastrin releasing peptide receptor
HCC	Hepatocellular carcinoma
HD	Huntington's disease
HR LBCL	High risk large B-cell lymphoma
IA	Interim analysis
iAMD	Intermediate age-related macular degeneration
IC-MPGN	Immune complex membranoproliferative glomerulonephritis
IgAN	IgA nephropathy
IPF	Idiopathic pulmonary fibrosis
ITP	Immune thrombocytopenia
LBCL	Large B-cell lymphoma
LN	Lupus nephritis
mCRPC	Metastatic castration-resistant prostate cancer
MDS	Myelodysplastic syndrome
mHSPC	Metastatic hormone sensitive prostate cancer
mPDAC	Metastatic pancreatic ductal adenocarcinoma
MS	Multiple sclerosis
NASH	Non-alcoholic steatohepatitis
nmCRPC	Non-metastatic castration-resistant prostate cancer
NPR1	Natriuretic peptide receptor 1
nr-axSpA	Non-radiographic axial spondyloarthritis
NSAI	Non-steroidal aromatase inhibitor
NSCLC	Non-small cell lung cancer
OS	Overall survival
PFS	Prefilled syringe
PNH	Paroxysmal nocturnal haemoglobinuria
PsA	Psoriatic arthritis
rHR	Resistant hypertension
rMS	Relapsing multiple sclerosis
rPFS	Radiographic progression free survival
SLE	Systemic lupus erythematosus
SMA Type 1	Spinal muscular atrophy (IV formulation)
SMA Type 2/3	Spinal muscular atrophy (IT formulation)
SpA	Spondyloarthritis
T1DM	Type 1 Diabetes mellitus
wAIHA	Warm autoimmune hemolytic anemia

References

Cosentyx®

1 Matusiak Ł. Br J Dermatol. 2020;183(6):e171-e177.
2 G6 market estimations based on IQVIA PADDS 2021.
3 Kimball A, et al. N Engl J Med. 2016;375:422–434.
4 Data on file. IQVIA PADSS. Novartis Pharmaceuticals Corp; March 2023.
5 Kimball A, et al. Lancet. 2023;401(10378):747-761.
6 Post hoc analysis: patients with moderate to severe pain at baseline who improved to mild or no pain at Week 52.
7 Novartis data on file. SUNNY Clinical Study Program pooled data tables and post hoc analyses.
8 Between 1 in 100 and 1 in 1,000 exposed patients.

Kesimpta®

1 March 2023, IQVIA NPA (Kesimpta®) and IQVIA NPA adjusted by NSP (all others). B-cell therapies as portion of MS market in NBRx.
2 Refers to US unless otherwise stated.
3 Data on file.
4 The initial dosing period consists of 20 mg subcutaneous doses at Weeks 0, 1 and 2, thereafter once a month. Patient must take pen out of the refrigerator 15-30 minutes before self-administering.
5 Efficacy outcomes as measured by disability progression and brain volume change.
6 Cohen et al, Poster presented at American Academy of Neurology, Boston, 22-27 April 23.
7 Cohen et al, oral presentation at American Academy of Neurology, Boston, 22-27 April 23.

Zolgensma®

1 Based on US SMA incidence from NBS data & Zolgensma sales in eligible patients.
2 Wave 3 and 4 launch countries.
3 Mendell J. et al. Long-Term Follow-Up of Onasemnogene Abeparvovec Gene Therapy in Symptomatic Patients with Spinal Muscular Atrophy Type 1. Abstract presented at the 2023 MDA Clinical & Scientific Conference. March 19-22,2023.
4 Connolly A. et al. Intravenous and Intrathecal Onasemnogene Abeparvovec Gene Therapy in Symptomatic and Presymptomatic Spinal Muscular Atrophy: Long-Term Follow-Up Study. Abstract presented at the 2023 MDA Clinical & Scientific Conference. March 19-22, 2023.
5 All but one achieved walking alone milestone before or without added therapy at the last data cut (May 2022).